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PTC Therapeutics Expands Access For Translarna™ (Ataluren)

-Italian Medicines Agency (AIFA) approves cohort early access program -

SOUTH PLAINFIELD, N.J., Nov. 3, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Translarna™ (ataluren) has been approved to be included in the list of drugs reimbursable by the Italian National Health System under Law 648/96. The decision is expected to be formalized through its publication in the official Italian journal, *Gazzetta Ufficiale* in the coming weeks. A list of drugs reimbursable under this law is published on the Italian Medicines Agency's (AIFA) website.

Filippo Buccella, President of Parent Project Onlus, said, "The story of Translarna, which began in 1998, has taught us a lot about Duchenne muscular dystrophy and the potential for new agents to treat the underlying cause of the disease. We are delighted that AIFA's decision means we can now bring the first therapy approved for nonsense mutation DMD to patients and families in Italy."

AIFA's Technical and Scientific Committee gave a positive opinion for the inclusion of Translarna to be available and reimbursed to patients at its recent October meeting. The approval is based on the request of specialist physicians and Parent Project Onlus to gain access to Translarna prior to its commercial availability in Italy. Law 648/96 allows a cohort of patients access to drugs which have demonstrated clear benefit while under clinical investigation, including drugs that have obtained a marketing authorization abroad but are not yet marketable in Italy. Translarna has been approved in the European Economic Area but is not yet commercially available in Italy.

"We are very pleased with the positive opinion from AIFA", stated Mark Rothera, Chief Commercial Officer of PTC Therapeutics, Inc. "DMD is a devastating and a rapidly progressing muscle wasting disorder and every day counts. We are committed to working with regulators, payors and the DMD community to enable Translarna to reach all patients who may benefit as soon as possible wherever reimbursed EAP mechanisms exist."

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. 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 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.

Forward Looking Statements:

Any statements in this press release about future expectations, plans and prospects for PTC, the development of and potential market for PTC's products and product candidates, our clinical trials, our current and planned regulatory submissions, our earlier stage programs, the potential advantages of Translarna 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 timing and conduct of clinical trials, the availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our products or 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. In addition, the forward-looking statements included in this press release represent PTC's views only as of the date of this press release. 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.

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