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Translarna Granted Orphan Drug Designation in the U.S. and Europe for the Treatment of Mucopolysaccharidosis I

SOUTH PLAINFIELD, N.J., Dec. 19, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (Nasdaq: PTCT) today announced that both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have granted orphan-drug designation (ODD) to Translarna™ (ataluren) for the treatment of patients with Mucopolysaccharidosis I (MPS I). MPS I is an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of by-products in the body's cells. There is significant unmet medical need and new treatments targeting the underlying cause of the disease are needed. MPS I represents the third indication for which Translarna has received orphan-drug designation. Translarna also has orphan-drug designation for Duchenne muscular dystrophy and cystic fibrosis.

Translarna is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. In preclinical models of MPS I, Translarna demonstrated that it crosses the blood-brain barrier and penetrates skeletal and cardiac tissues. The same preclinical models indicate that Translarna treatment reduces GAG levels in multiple tissues with no observed signs of cell toxicity or stress. Since Translarna is administered systemically, crosses the blood-brain barrier, and penetrates other tissues relevant to disease, there is potential for Translarna treatment to address the cardiac and neurological defects associated with nonsense mutation MPS I.

"The receipt of orphan-drug designation in MPS I from both the FDA and the EMA further supports the broad potential applicability of Translarna across numerous nonsense mutation based disorders," stated Stuart Peltz, Ph.D., CEO of PTC Therapeutics, Inc. "Our goal is to continue to pursue the development of this therapy for DMD, CF and MPS I, and other indications in order to help treat conditions where there is high unmet medical need. With over twenty publications by independent investigators demonstrating Translarna's activity across numerous pre-clinical nonsense mutation-based models, Translarna truly has the potential to be a pipeline within a product. We look forward to confirming this activity through additional clinical proof-of-concept studies beginning in 2015."

In the U.S., Orphan drug designation is granted by the FDA's Office of Orphan Products Development to promote the development of products that may offer therapeutic benefits for diseases with a prevalence of fewer than 200,000 individuals per year. Orphan-drug designation provides opportunities for grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States, if granted FDA approval.

Similarly, EMA's Orphan Medicinal Product Designation is designed to promote the development of drugs that may provide significant benefit to patients suffering from rare, life-threatening diseases. In addition to 10 years of market exclusivity if Translarna is approved for the treatment of nonsense mutation MPS I, the designation also provides special incentives for sponsors including eligibility for protocol assistance and possible exemptions or reductions in certain regulatory fees during development or at the time of application for marketing approval.

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 Mucopolysaccharidosis I (MPS I)

MPS I is an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of byproducts of chemical reactions in the body's cells. Globally, MPS I occurs in about 1 in every 100,000 births. It is estimated that 60 percent to 80 percent of cases of MPS I are caused by a nonsense mutation. There is no cure for MPS I and enzyme replacement therapies do not sufficiently address the central nervous system, skeletal or cardiac symptoms associated with the

disorder. Prognosis of patients with MPS I is poor and there is an urgent need for the development of new treatments targeting the underlying cause of MPS I.

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 conduct of our clinical trials of Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy, cystic fibrosis and MPS I, caused by nonsense mutations, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available; our plans to pursue development of Translarna for additional indications other than Duchenne muscular dystrophy, cystic fibrosis and MPS I, caused by nonsense mutations; the benefits that may be recognized in Europe and the United States in the event that Translarna is approved for the treatment of MPS I; 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," "opportunity," "possible," "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/translarna-granted-orphan-drug-designation-in-the-us-and-europe-for-the-treatment-of-mucopolysaccharidosis-i-300012390.html

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