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PTC Therapeutics Submits European Application For Translarna™ for Cystic Fibrosis

- First potential disease-modifying treatment for people with nonsense mutation cystic fibrosis, the most severe form of the disease -

SOUTH PLAINFIELD, N.J., Sept. 30, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that the European Medicines Agency (EMA) has validated the submission of a variation for a new indication for Translarna™ (ataluren for the treatment of nonsense mutation cystic fibrosis (nmCF) for patients not taking chronic inhaled aminoglycoside antibiotics. If approved, Translarna would be the first oral protein restoration treatment that targets the underlying cause of nmCF. Approximately 10 percent of cystic fibrosis patients have their disease as a result of a nonsense mutation, which can cause the most severe form of cystic fibrosis. Translarna received marketing authorization in the European Economic Area in July 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged five years and older.

"We are very excited to be advancing our second indication, which represents another milestone in realizing the full potential for Translarna." said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are eager to bring Translarna to patients with nonsense mutation cystic fibrosis, who currently have no other treatment options for the underlying cause of their disorder. We look forward to working with regulators to help bring this precision based medicine to patients as quickly as possible."

"Nonsense mutation cystic fibrosis is a serious form of cystic fibrosis and is very challenging to treat. We are pleased that there may be a treatment option for people with nmCF in the near future," said Jacquelien Noordhoek, President of CF Europe. "The progress that is being made to better understand and treat the underlying genetic causes of cystic fibrosis is critical and must continue."

The regulatory application for Translarna for nmCF is based on previously announced analyses from the company's prior completed Phase 3 double-blind, placebo-controlled study comparing Translarna to placebo in nmCF patients. PTC is conducting an additional randomized, double-blind, placebo-controlled Phase 3 study of Translarna in nmCF patients and expects enrollment to be completed by the end of this year, with top-line data expected by the end of 2016.

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.

The FDA and the European Commission have granted Translarna Orphan Drug status for the treatment of nonsense mutation cystic fibrosis and nonsense mutation Duchenne muscular dystrophy.

Translarna has the potential to benefit patients across the vast array of genetic disorders caused by a nonsense mutation. On average, 11% of every monogenic disorder is caused by a nonsense mutation. PTC's strategy is to rapidly expand the clinical development of Translarna across multiple genetic disorders to deliver on the company commitment to address rare and neglected disorders.

About Cystic Fibrosis
Cystic fibrosis (CF) is a rare disabling and life-threatening genetic disorder resulting from mutations that cause the lack or dysfunction of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, a chloride ion transport channel which results in thick mucus secretions in the lung, pancreas and other organs. In patients who have cystic fibrosis due to a nonsense mutation, an interruption in the genetic code prematurely halts the synthesis of CFTR, causing the protein to be short and non-functioning. Nonsense mutations are categorized as Class I mutations that result in little or no production of the

CFTR protein, which makes people with these mutations very difficult to treat. CF patients with Class I mutations typically experience more severe disease symptoms than those with other genotypes, including a shorter life span, a higher probability of end-stage lung disease, and a higher prevalence of pancreatic insufficiency. Approximately 10% of patients have CF due to a Class I nonsense mutation in at least one allele of the CFTR gene. Available therapies for treatment of lung manifestations of CF, such as inhaled antibiotics do not address the underlying defect. There are no marketed treatments that target the defect associated with CF caused by nonsense mutations.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology 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 are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the rate and degree of market acceptance and clinical utility of Translarna; PTC's estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; the timing and conduct of PTC's clinical trial of Translarna for the treatment of nmCF, including statements regarding the timing of initiation, evaluation, enrollment and completion of the trial and the period during which the results of the trial will become available; matters related to our current and planned regulatory filings, including timing for EMA review of our variation submission for nmCF, whether the EMA will grant a positive opinion with respect to such submission, and the timing and determinations of the European Commission following any issuance of the EMA's opinion; our strategy, future operations, future financial position, future revenues or projected costs; and objectives of management. Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to the EMA's determinations with respect to our nmCF variation submission ; the initiation, conduct and availability of data from clinical trials and studies; expectations for regulatory approvals; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates and the factors discussed in the "Risk Factors" section of PTC's 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 SEC. 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 PTC does 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.