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PTC Completes Enrollment of Phase 3 Clinical Trial of Translarna™ for Patients with Cystic Fibrosis

SOUTH PLAINFIELD, N.J., Nov. 19, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has completed enrollment of ACT CF, the company's second Phase 3 clinical trial of Translarna™ (ataluren) for patients with nonsense mutation cystic fibrosis (nmCF). Nonsense mutations in cystic fibrosis are categorized as Class I mutations, which are the most difficult to treat, as they result in little or no production of the cystic fibrosis transmembrane conductance regulator (CFTR) protein. Approximately 10 percent of cystic fibrosis patients have their disease as a result of a nonsense mutation.

"We are very pleased by the enthusiastic response from our clinical investigators and strong interest from patients to participate in our ACT CF study," said Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Based on the results from our previous Phase 3 study, we believe that Translarna has the potential to meaningfully improve lung function and decrease pulmonary exacerbations in nonsense mutation cystic fibrosis patients by targeting the underlying cause of the disease."

On September 30, 2015, the European Medicines Agency (EMA) validated the submission of a variation for a new indication for Translarna for the treatment of nmCF. The company's regulatory application for Translarna in nmCF was based on clinical data and analyses generated from the company's previously completed Phase 3 double-blind, placebo-controlled study comparing Translarna to placebo in nmCF patients. Translarna received marketing authorization in Europe in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged five years and older.

About ACT CF
ACT CF is a 48-week placebo controlled Phase 3 clinical trial designed to evaluate the effect of Translarna in patients six years of age or older with nmCF not receiving chronic inhaled aminoglycosides. The primary endpoint is lung function as measured by relative change in percent predicted FEV1. The study has reached full enrollment across 88 sites globally. Patients in the trial are eligible to participate in an open-label extension study, which has already begun enrolling patients who have completed the initial 48 weeks of treatment.

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 following indications: Duchenne muscular dystrophy, cystic fibrosis, Mucopolysaccharidosis I (MPS 1), and aniridia.

Translarna is an oral protein restoration therapy that has the potential to benefit patients with 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 expand the clinical development of Translarna across multiple genetic disorders to deliver on the company's 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), a chloride ion channel function responsible for fluid transport 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 timing, results and conduct of PTC's clinical trials and studies of Translarna for the treatment CF, MPS I and aniridia caused by nonsense mutation, including statements regarding the timing of initiation, evaluation, enrollment and completion of the trials and studies and the period during which the results of the trials and studies will become available; the clinical utility and potential advantages of Translarna; the rate and degree of market acceptance; 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 of PTC's planned regulatory filings, including with the FDA, the EMA and other regulatory bodies outside of the United States and European Economic Area, or EEA; 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 outcome of final analyses of the data from the Company's Phase 3 clinical trial in nmDMD, or ACT DMD, which may vary from PTC's initial analysis or lead to different (including more or less favorable) interpretations of the results than the analyses conducted to date, or both; whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), adverse decisions by regulatory authorities, other delay or deceleration of the regulatory process, and PTC's ability to meet existing or future regulatory standards with respect to Translarna; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; PTC's ability to maintain the marketing authorization of Translarna for the treatment of nonsense mutation DMD in the EEA, which is conditioned upon, among other things, completion of ACT DMD and submission of the final report, including additional efficacy and safety data from ACT DMD, during 2015 and which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to obtain marketing authorization for the treatment of nmCF in the EEA or elsewhere; PTC's ability to commercialize Translarna and commercially manufacture in general and specifically as a treatment for nonsense mutation DMD, including its ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which it may obtain regulatory approval, including the United States, EEA and other territories; the initiation, conduct and availability of data from clinical trials and studies; 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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/ptc-completes-enrollment-of-phase-3-clinical-trial-of-translarna-for-patients-with-cystic-fibrosis-300181506.html

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