June 30, 2014

PTC THERAPEUTICS INITIATES CONFIRMATORY PHASE 3 CLINICAL TRIAL OF TRANSLARNA™ (ATALUREN) IN PATIENTS WITH NONSENSE MUTATION CYSTIC FIBROSIS (NMCF)

SOUTH PLAINFIELD, NJ – JUNE 30, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the initiation of a global confirmatory Phase 3 clinical trial of Translarna™ (ataluren), an investigational new drug, in patients with nonsense mutation cystic fibrosis (nmCF). Nonsense mutations within cystic fibrosis are categorized as Class I mutations, a severe form of CF that results in little or no production of the CFTR protein. The Phase 3 confirmatory trial is referred to as ACT CF (ataluren confirmatory trial in cystic fibrosis) and the primary endpoint is lung function as measured by relative change in percent predicted forced expiratory volume in one second, or FEV1.

"We believe the data from our previous 238-patient Phase 3 clinical trial in nonsense mutation cystic fibrosis patients demonstrated that Translarna had a positive benefit on lung function versus placebo, particularly in patients not receiving chronic inhaled tobramycin. The ACT CF trial is designed to confirm Translarna's efficacy based on the evidence seen in the previous Phase 3 study and other earlier work. By focusing ACT CF on the patient population that can most readily demonstrate the effect of Translarna, we believe we have optimized our opportunity for a successful trial," stated Robert Spiegel, M.D., FACP, Chief Medical Officer of PTC Therapeutics. "We look forward to completing ACT CF and ultimately bringing this potential first-in-class treatment to those CF patients who may benefit."

"The CF community is excited by the start of this pivotal study in cystic fibrosis," stated Michael Konstan, M.D., Pediatric Department Chair at University Hospitals Rainbow Babies & Children's Hospital and Professor of Pediatrics at Case Western Reserve University School of Medicine. "Current treatments for nonsense mutation cystic fibrosis focus on alleviating symptoms and reducing infections, whereas Translarna™ targets the underlying cause of disease. There is a significant need for better treatment options for patients with this severe form of the disease, and we believe Translarna™ has the potential to provide this benefit."

ABOUT ACT CF
ACT CF is an international, randomized, double-blind, placebo-controlled, efficacy and safety study of Translarna™ (ataluren 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 is planning to enroll 208 patients. Patients are randomly assigned to one of two treatment arms: Translarna™ (ataluren) three times per day (40 mg/kg/day) or placebo (morning, midday, evening). It is anticipated that participants who have successfully completed this study will have the opportunity to receive Translarna™ (ataluren) in an extension study, except in countries where Translarna™ (ataluren) is commercially available for the treatment of nonsense mutation cystic fibrosis.

ABOUT CYSTIC FIBROSIS
Cystic fibrosis (CF) is a disabling and life-threatening autosomal recessive disorder resulting from mutations that cause dysfunction in the cystic fibrosis transmembrane conductance regulator (CFTR). In nonsense mutation cystic fibrosis, an interruption in the genetic code - known as a nonsense mutation - 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. 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 TRANSLARNA™ (ATALUREN)
Translarna™ (ataluren), an investigational new drug discovered and developed by PTC Therapeutics, 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 CFTR in nonsense mutation cystic fibrosis. The development of Translarna™ (ataluren) 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. In May 2014, Translarna™ received a positive opinion for conditional approval by the CHMP for patients with nonsense mutation Duchenne muscular dystrophy in ambulatory boys who are five years of age and older. The CHMP opinion will form the basis for a European Commission (EC) decision as to whether to formally grant the conditional marketing authorization. The European Commission will review the positive opinion from the CHMP and generally delivers its final decision within three months.

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, are forward-looking statements including statements concerning the future expectations, plans and prospects for PTC Therapeutics, Inc. (PTC); the timing and conduct of ACT CF, including statements regarding anticipated enrollment and timing; the potential advantages of Translarna (ataluren); the timing of regulatory approvals, including any determination (whether positive or negative) by the European Commission (EC) with respect to conditional marketing authorization for Translarna in nonsense mutation Duchenne muscular dystrophy (nmDMD); and the objectives of management. Other forward-looking statements may be identified by the words "anticipate," "believe," "look forward," "expect," "intend," "may," "plan," "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, among others, those related to the initiation and conduct of clinical trials; availability of data from clinical trials; expectations for regulatory approvals (including the EC's determination with respect to conditional marketing authorization for Translarna in nmDMD); our scientific approach and general development progress; the availability or commercial potential of our product candidates; and other factors discussed in the "Risk Factors" section of PTC's most recent Form 10-Q and 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 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.