June 10, 2016

# New Analyses from Phase 3 Study 009 of Translarna™ (ataluren) in Patients with Cystic Fibrosis Presented at 39th European Cystic Fibrosis Conference

SOUTH PLAINFIELD, N.J., June 10, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that new analyses from the company's previous Phase 3, double-blind, placebo-controlled, 48-week 009 trial of Translarna™ (ataluren) for the treatment of nonsense mutation cystic fibrosis (caused by at least one nonsense mutation) are being presented at the 39th European Cystic Fibrosis Conference, June 8-11, 2016, in Basel, Switzerland.

For more information, visit https://www.ecfs.eu/basel2016.

***Poster Presentations***

- Data regarding lung function (ePoster WS13.1.1) and exacerbations (ePoster WS13.1) from the Phase 3 study, as well as data regarding the natural history of patients with cystic fibrosis carrying nonsense mutations (Poster 264), are being presented during poster sessions beginning June 9th, 2016.

***Oral Presentation***

- Clinical benefits of ataluren in patients with CF and nonsense mutations, Friday, June 10th, 2016, 3:00 p.m. - 4:30 p.m. presented by J. Davies, Royal Brompton and Harefield NHS Imperial College London, London, UK

**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 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 is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. 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:**
All statements, other than those of historical fact, contained in this press release, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the clinical utility and potential advantages of Translarna; the rate and degree of market acceptance of Translarna; the timing, results and conduct of PTC's clinical trials and studies of Translarna; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "will," "work towards," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "plan," "potential," "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 timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), including whether PTC is required to perform additional clinical and non-clinical trials at significant cost and whether such trials, if successful, may enable FDA review of a new drug application (NDA) submission; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the EMA determines that the risk-benefit balance of Translarna supports continuation of our marketing authorization in the EEA on a conditional basis, a full basis, or at all; whether other regulators agree with PTC's interpretation of the results of ACT DMD; the EMA's determinations with respect to PTC's variation submission which seeks to add Translarna for the treatment of nonsense mutation cystic fibrosis to PTC's marketing authorization in the EEA; 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 commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna, including whether final guidance from National Institute for Health and Care Excellence (NICE) recommends Translarna for the treatment of nmDMD and the acceptability of final terms of any market access agreement between PTC and NHS England; whether patients and healthcare professionals may be able to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome, including whether Translarna may be accessed through a reimbursed importation pathway provided under German law and whether such pathway will be utilized by German patients while maintaining a sustainable price for Translarna; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), the period during which the outcome of regulatory reviews will become available, 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; PTC's ability to fulfill any additional obligations, including with respect to further trials or studies relating to cost-effectiveness, obtaining licenses or satisfying requirements for labor and business practices, in the territories 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; the outcome of ongoing or future clinical trials or studies; PTC's ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; the sufficiency of PTC's cash resources and PTC's ability to obtain adequate financing in the future for PTC's foreseeable and unforeseeable operating expenses and capital expenditures; 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.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

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 press release except as required by law.