April 15, 2016

NICE Recommends Translarna™ (ataluren) for the Treatment of Patients with Nonsense Mutation Duchenne Muscular Dystrophy in England

-Positive Recommendation Enables Reimbursed Patient Access to First Approved Therapy to Treat Underlying Cause of Devastating Rare Disease-

SOUTH PLAINFIELD, N.J., April 15, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that the National Institute for Health and Care Excellence (NICE) has recommended Translarna ∇ (ataluren) for ambulatory patients aged five years and older with nonsense mutation Duchenne muscular dystrophy (nmDMD) in connection with a Managed Access Agreement (MAA) with NHS England. The provision of patient access is subject to the finalization of the NICE draft guidance, which the agency expects in May of 2016.

"This is an important day for the Duchenne community, which has been working hard to make this ground-breaking drug available to boys with nonsense mutation Duchenne muscular dystrophy," said Paul Lenihan, Chief Executive of Action Duchenne. "We are delighted by this positive recommendation and NICE's recognition that Translarna is an important new medicine for patients. This decision is a hugely encouraging sign that both NICE and NHS England have listened to the patient community, bringing hope to each and every parent and patient fighting DMD."

Primarily affecting males, Duchenne muscular dystrophy (DMD) is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of skeletal, diaphragm, and heart muscles. Patients with DMD lose the ability to walk from as early as 10 years of age and experience life-threatening lung and heart complications in their late teens and early twenties.

"We are extremely pleased by the NICE recommendation, which recognizes Translarna as an innovative medicine with the potential to change the course of this devastating disease," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This is a crucial step for the boys and young men in England with nonsense mutation DMD. We are grateful to the patients, families, advocacy groups and physicians who have supported PTC Therapeutics through this important access process and look forward to working with NHS England to conclude the managed access agreement."

PTC and NHS England are in the process of finalizing an MAA outlining financial and clinical details surrounding the use of Translarna, including a confidential financial arrangement. The MAA is expected to allow PTC to collect further data on the efficacy of Translarna for the treatment of nmDMD over a five-year period with NICE guidance to be reviewed again at the end of that period.

Translarna was approved by the European Commission in August 2014 to treat nmDMD and is currently available to patients in 23 countries through either expanded access programs or commercial sales.

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.

Further information about Translarna, including the European Public Assessment Report, Summary of Product Characteristics and Patient Information Leaflet, is available on the European Medicines Association website.

This medicinal product is subject to additional monitoring. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system or to PTC at medinfo@ptcbio.com.

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, 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 about our expectations regarding the potential timing and outcome of final guidance from NICE with respect to Translarna for the treatment of nmDMD; the ability of NHS England and PTC Therapeutics to finalize a MAA in a timely manner, whether on terms acceptable to PTC, or at all; the potential timing, if ever, of the provision of patient access to Translarna for nmDMD in England; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance of Translarna; the clinical utility and potential advantages 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; future expectations, plans and prospects for PTC; 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 "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: whether final guidance from NICE recommends Translarna for the treatment of nmDMD; actual reimbursement decisions by NHS England and the acceptability of final terms of the MAA; PTC's ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, or EEA, which is subject to ongoing conditions and is also subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, 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; the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of 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 NDA submission; whether the FDA, the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD and other data with respect to the safety and efficacy of Translarna for the treatment of nmDMD or PTC's other clinical trials; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; 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 minimize any access issues for German patients while maintaining a sustainable price; expectations for regulatory approvals, including PTC's ability to make or advance 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; 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 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; 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 Annual Report on Form 10-K 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 release except as required by law.

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