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NHS England Enables Access to Translarna™ ▼ (ataluren) For Patients with Nonsense Mutation Duchenne Muscular Dystrophy

--Important decision allows reimbursed access to Translarna, the first approved therapy to treat the underlying cause of Duchenne muscular dystrophy--

SOUTH PLAINFIELD, N.J., July 7, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the company and NHS England have successfully negotiated a Managed Access Agreement (MAA) for Translarna (ataluren) for ambulatory patients aged five years and older with nonsense mutation Duchenne muscular dystrophy (nmDMD). This decision provides reimbursed patient access to Translarna in England via a five-year MAA. Translarna previously received a positive recommendation from the National Institute for Health and Care Excellence (NICE) in April of 2016, subject to PTC and NHS England finalizing the terms of the MAA. NICE is expected to issue final guidance later this month following execution of the MAA, with implementation soon after.

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.

"This is an important day in England for children and young adults suffering from DMD," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are extremely pleased to have reached a successful outcome with NHS England, which will provide long-awaited access to Translarna for patients with nonsense mutation DMD. We are grateful to the patients, families, advocacy groups and physicians for their tremendous effort in supporting PTC Therapeutics throughout this important and rigorous access process."

PTC and NHS England have now finalized the outstanding aspects of the MAA which include a confidential financial arrangement and the collection of 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, before future funding decisions are taken.

Translarna received marketing authorization from the European Commission to treat nmDMD in August 2014, which is currently under annual review by the European Medicines Agency with an opinion on renewal expected mid-2016. Translarna is currently available to patients in more than 20 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 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; timing and outcome of PTC's regulatory strategy and process, including the execution of the MAA, issuance of final guidance from NICE with respect to Translarna, the implementation of such guidance in England, and the European Medicines Agency's (EMA) opinion with respect to the potential renewal or approval of the marketing authorization for Translarna in nmDMD in the European Economic Area (EEA) and any restrictions or conditions that may be placed on any such renewal or approval; PTC's ability to maintain its current marketing authorizations, including its ability to satisfy any obligations or conditions that may be placed on its marketing authorization in the EEA, or obtain and maintain additional marketing authorizations; the clinical utility and potential advantages of Translarna; PTC's ability to continue to supply Translarna to patients across Europe and in other territories; 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," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "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: expectations with respect to execution of the MAA, whether final guidance from NICE recommends Translarna for the treatment of nmDMD; actual reimbursement decisions by NHS England; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the EMA's Committee for Medicinal Products for Human Use determines that the benefit-risk balance of Translarna supports renewal or approval of PTC's marketing authorization in the EEA and any restrictions or conditions that may be placed on any such renewal or 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 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; 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; 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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/nhs-england-enables-access-to-translarna--ataluren-for-patients-with-nonsense-mutation-duchenne-muscular-dystrophy-300295263.html

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