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CHMP Recommends Renewal of Translarna's™ Marketing Authorization for Nonsense Mutation Duchenne Muscular Dystrophy Based on Continued Positive Benefit-Risk Assessment

--PTC to conduct a post-authorization clinical trial --

SOUTH PLAINFIELD, N.J., Nov. 11, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has recommended the renewal of the conditional marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients five years and older. In connection with the renewal, the marketing authorization will include a specific obligation to conduct an additional long-term post-authorization trial.

"We are pleased with this outcome which took into account all available data for Translarna," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This decision reflects the benefit that Translarna is having for patients suffering from nonsense mutation Duchenne muscular dystrophy."

The CHMP opinion forms the basis for a European Commission decision on the renewal of the marketing authorization. The European Commission generally delivers its decision within three months.

"Translarna has shown clinically meaningful benefits for patients," said Eugenio Mercuri, M.D., Professor of Pediatric Neurology at the Catholic University, Rome, Italy. "Duchenne is a devastating disease with a progressive loss of function. Maintaining function is of the utmost importance to patients."

"The consistency of Translarna's benefit shown across key endpoints is impressive for a dystrophin replacement therapy," said Craig McDonald, M.D., Professor of Pediatrics and Chair of the Department of Physical Medicine & Rehabilitation at University of California. "I am encouraged for the DMD community by the CHMP's recommendation."

The CHMP has requested that PTC conduct a new 18-month randomized, placebo-controlled study in patients with nonsense mutation Duchenne muscular dystrophy, as a specific post-authorization obligation, with results expected to be available in the first quarter of 2021. This study will be followed by an 18-month open-label extension period where all patients will be switched to Translarna. PTC has proposed a trial similar in size to ACT DMD and details of the protocol are expected to be finalized in future interactions with the EMA. Conditional marketing authorizations are subject to annual reassessment and renewal.

"For boys with Duchenne, every day matters and functional loss cannot be regained. Patients need access to innovative new therapies like Translarna," stated Filippo Buccella, founder of the Italian Parent Project, a patient advocacy group for Duchenne Muscular Dystrophy.

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 Duchenne Muscular Dystrophy
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, the more severe form of the disorder, lose the ability to walk as early as age 10 and experience life-

threatening lung and heart complications in their late teens and twenties. It is estimated that a nonsense mutation is the cause of DMD in approximately 13 percent of patients.

About PTC Therapeutics
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 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.

For More Information:

Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

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 timing and outcome of PTC's regulatory process, including the final determination by the European Commission with respect to renewal of the marketing authorization in the European Economic Area (EEA) for Translarna for the treatment of nmDMD; the final design, enrollment, timing, conduct, cost, evaluation and results of the clinical trial of Translarna for the treatment of nmDMD that PTC will undertake pursuant to the specific obligation associated with the Translarna marketing authorization (if renewal is granted by the European Commission); 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 "will," "plan," "target," "anticipate," "believe," "estimate," "expect," "intend," "may," "potential," "project," "possible," "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: PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Commission determines to approve the renewal of such authorization and whether the EMA determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; the final design of the new nmDMD trial that PTC will undertake pursuant to the specific obligation associated with the marketing authorization (following renewal) and PTC's ability to enroll, fund and conduct such trial; the 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 New Drug Application (NDA) submission; 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; the outcome of ongoing or future clinical trials or studies in Translarna, including ACT CF and the Phase 2 study of Translarna for nmDMD in pediatric patients; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, including its ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; 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 (or 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; PTC's scientific approach and general

development progress; 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/chmp-recommends-renewal-of-translarnas-marketing-authorization-for-nonsense-mutation-duchenne-muscular-dystrophy-based-on-continued-positive-benefit-risk-assessment-300361366.html

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