July 25, 2016

PTC Therapeutics Provides Regulatory Update on Translarna™ (ataluren)

SOUTH PLAINFIELD, N.J., July 25, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced global regulatory updates on Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) and nonsense mutation cystic fibrosis (nmCF).

European update on Translarna for nmDMD

Over the last several months, PTC has been engaged in constructive discussions with the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) regarding the renewal of Translarna's marketing authorization. The company has been informed that the renewal assessment procedure cannot be completed by mid-year 2016, however PTC expects that Translarna's current marketing authorization status will remain valid until a decision is adopted by the European Commission. The CHMP has agreed to the proposal by PTC to submit a draft clinical trial protocol for further discussion, which includes seeking scientific advice from the EMA. The company is optimistic that these interactions will support the renewal of the marketing authorization of Translarna coupled with an obligation to conduct an agreed upon clinical trial.

"We appreciate all the effort put forth by the CHMP in this renewal process," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are pleased that meanwhile patients will continue to have access to Translarna, the first-ever treatment approved for an underlying cause of Duchenne muscular dystrophy."

Translarna initially received marketing authorization in the European Economic Area for the treatment of nmDMD in 2014, subject to annual renewal and certain conditions. Translarna is currently available to patients in more than 20 countries outside of the U.S. commercially or through early access programs.

Phase 2 Clinical Study of Translarna for nmDMD in pediatric patients
As part of PTC's ongoing commitments under the European marketing authorization and to support the potential expansion of Translarna's label to younger patients, the company has initiated a pediatric clinical study of Translarna for the treatment of nmDMD in patients two to five years of age. This Phase 2, open-label, multiple-dose study will evaluate the safety and pharmacokinetics of Translarna in pediatric patients.

National Institute for Health and Care Excellence (NICE) guidance update
The National Institute for Health and Care Excellence (NICE) recently issued final guidance recommending Translarna for the treatment of nmDMD patients in England in connection with a Managed Access Agreement (MAA) with National Health Services England (NHS). As part of the MAA, the usual three-month funding period under local regulation has been waived by NHS, meaning Translarna could be available within weeks to nmDMD patients in England.

U.S. update on Translarna for nmDMD
As previously disclosed, PTC recently participated in discussions with the U.S. Food and Drug Administration (FDA) to discuss the Refuse to File (RTF) letter issued on February 22, 2016 with respect to the company's New Drug Application (NDA) for Translarna for the treatment of nmDMD. PTC recently submitted an appeal to escalate continuing discussions about the RTF decision to the next level of FDA management via the formal dispute resolution process within FDA's Center for Drug Evaluation and Research (CDER). This process exists to encourage open, prompt discussion of scientific and procedural disputes that arise during the drug development process between FDA and companies. Within the dispute resolution process, PTC is willing to consider multiple paths to advance a potential FDA approval, including the possibility of conducting an additional clinical trial under accelerated approval.

European update on Translarna for nmCF
During the third quarter of 2015, PTC submitted a variation to its marketing authorization requesting EMA approval of Translarna for the treatment of nmCF based on a post-hoc analysis of the company's previously completed Phase 3 clinical trial. Based on recent interactions with the CHMP, PTC no longer anticipates that the CHMP will issue its opinion regarding this submission in mid-2016. As previously communicated, PTC's confirmatory Phase 3 ACT CF trial is currently ongoing, and there is substantial risk that the results from this trial, expected in early 2017, will be required for approval.

PTC to Host Conference Call to Discuss Second Quarter Financial Results
PTC will host a webcast conference call to report its second quarter financial results and provide an update on the company's business and outlook on Thursday, August 4, 2016 at 4:30 p.m. (ET) after the closing of the market.

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 EMA scientific advisors
The EMA scientific advisors include the Scientific Advice Working Party (SAWP) and the Scientific Advisory Group (SAG). The SAWP is a standing working party established by the CHMP with the sole remit of providing scientific advice and protocol assistance. The SAG is convened at the request of the CHMP to provide independent recommendations on scientific or technical matters relating to products under evaluation by the CHMP, or on any other scientific issue relevant to the work of the CHMP.

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.

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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 timing and outcome of PTC's regulatory strategy and process, including (i) when the EMA's CHMP will issue an opinion with respect to the renewal of the marketing authorization for Translarna for the treatment of nmDMD and, when issued, whether such opinion will be positive, (ii) the nature of any conditions or restrictions that may be placed on any renewal of the marketing authorization by the European Commission, (iii) PTC's ability to design an acceptable new clinical trial in nmDMD with input from the EMA's advisors, (iv) PTC's ability to resolve with the FDA the matters set forth in the Refuse to File letter or otherwise advance Translarna for the treatment of nmDMD in the United States, whether pursuant to the formal dispute resolution process, an accelerated approval process, or otherwise, (v) whether PTC's Phase 2 study of Translarna for nmDMD in pediatric patients may support expansion of Translarna's label to younger patients, and (vi) when Translarna will be available to nmDMD patients in England; 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," "optimistic," "anticipate," "believe," "consider," "expect," "intend," "may," "potential," "project," "possible," "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: PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the EMA determines that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA; the nature and scope of any new nmDMD trial that PTC may design with the input of the EMA 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 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, 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, 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 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/ptc-therapeutics-provides-regulatory-update-on-translarna-ataluren-300302920.html

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