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PTC Therapeutics Provides Regulatory Update on Translarna™ (ataluren) for Nonsense Mutation Duchenne Muscular Dystrophy

SOUTH PLAINFIELD, N.J., Oct. 17, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a regulatory update on Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD).

U.S. regulatory update

PTC Therapeutics announced today that at the end of last week, the Office of Drug Evaluation I (ODE-I) of the U.S. Food and Drug Administration (FDA) denied the company's first appeal of the refuse to file letter issued by the FDA's Division of Neurological Products (DNP) on February 22, 2016 regarding PTC's New Drug Application (NDA) for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD).

The company intends to escalate its appeal to the next supervisory level of the FDA. This is an iterative process and the company anticipates that multiple cycles of appeals to progressively higher levels of the FDA may be required.

PTC continues to assert that a proper assessment of the data and analyses from multiple clinical studies, including two of the largest placebo-controlled trials ever conducted in DMD, can only be accomplished in the context of a full and fair review by the FDA. This would include an advisory committee meeting that allows clinical experts and representatives of the patient community to express their views on Translarna for the treatment of nmDMD. The company believes that Translarna is the only therapy in clinical development designed to target the underlying cause of nmDMD. In addition, a favorable safety profile has been consistently demonstrated in PTC's clinical trials, which have enrolled over 1,000 individuals to date.

"We believe that fair consideration of the totality of Translarna's data requires a full review of our application by the FDA," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "In light of this, continuing the formal dispute resolution process reflects our ongoing commitment to work with regulators and the Duchenne community to make Translarna available to nmDMD patients in the United States."

In addition, the company maintains its position that PTC should, under existing law and in fairness to patients, be provided the same opportunity for full review that the DNP gave to other recent applicants for products in development for different subsets of the DMD population.

"I am disappointed that a treatment for patients with nonsense mutation DMD is still not receiving a fair opportunity in front of the FDA," said Pat Furlong, President and Founder of Parent Project Muscular Dystrophy. "This inconsistency is unacceptable and is concerning for the entire community. This devastating, muscle-wasting disease cuts short the lives of boys and young men and every day that we wait for treatments, is a day in which muscle function is lost and not regained. As a community, we cannot rest until there are treatments for all the boys and young men."

European regulatory update

PTC recently participated in an oral explanation meeting before the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) in connection with the company's ongoing annual renewal of its marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. The marketing authorization in the European Economic Area (EEA), originally granted in August 2014, is subject to an annual renewal process, including an assessment by European regulators of a risk-benefit profile in favor of Translarna authorization.

Following conclusion of the recent oral explanation, the CHMP issued a request for supplemental information (RSI), including a request categorized as a major objection. Generally speaking, renewal of a marketing authorization requires a company to adequately address the points raised in a major objection. As with prior RSIs received by the company during this renewal process, the major objection relates to the efficacy and overall risk-benefit profile of Translarna as well as the design and conduct of an additional clinical trial that would provide comprehensive clinical data. The RSIs also include requests categorized as other concerns, which do not rise to the level of a major objection, and are generally associated with the primary pharmacology of Translarna and label matters.

The company continues to believe that if the CHMP issues a positive opinion in favor of the renewal of Translarna's marketing authorization, such renewal, and any subsequent annual renewals, will be coupled with an obligation to conduct an agreed upon new clinical trial of Translarna for the treatment of nmDMD. The EMA could also impose other new conditions to the authorization for renewal or make other recommendations, including the potential withdrawal of the marketing authorization.

PTC anticipates that an opinion regarding its marketing authorization renewal request will be adopted by the CHMP before the end of 2016. The company expects that its current marketing authorization will remain valid while the EMA's assessment is ongoing and until it is concluded with a decision from the European Commission.

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 per cent 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.

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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 (if such marketing authorization is renewed), (iii) PTC's ability to design an acceptable new clinical trial in nmDMD with input from the EMA, (iv) PTC's ability to resolve the matters set forth in the RSIs received to date from the

CHMP, and (v) the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including PTC's ability to resolve the matters set forth in the Refuse to File letter with the FDA or otherwise advance Translarna for the treatment of nmDMD in the United States (whether pursuant to the formal dispute resolution process or otherwise); the clinical utility and potential advantages 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 "potential," "expect," "believe," "plan," "anticipate," "estimate," "intend," "may," "possible," "will," "would," "could," "should," "continue," "project," "target," 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 EMA determines that the benefit-risk balance of Translarna authorization supports renewal of the company's marketing authorization in the EEA and whether the European Commission determines to renew such authorization; 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-for-nonsense-mutation-duchenne-muscular-dystrophy-300345673.html

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