PTC Provides Update on Translarna™ (ataluren) Application for Label Expansion

June 28, 2019

SOUTH PLAINFIELD, N.J., June 28, 2019 /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 completed their review of a proposed indication extension of Translarna™ (ataluren) for the treatment of patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) who are non-ambulatory. While the CHMP adopted a negative opinion of the extension, PTC was informed by EMA representatives that the European Public Assessment Report (EPAR) will be updated to clarify that patients who start Translarna while ambulatory are not required to discontinue treatment after loss of ambulation.

Translarna is currently indicated for ambulatory Duchenne patients who are over two years of age; the requested extension would have allowed for the inclusion of non-ambulatory patients in the label. PTC plans to request a re-examination of the procedure within the next two weeks and expects the new examination to last approximately 4 months.

"While we are disappointed with the current outcome of the label expansion procedure and its impact on non-ambulatory patients with nonsense mutation Duchenne Muscular Dystrophy, we are pleased that patients on Translarna can remain on treatment after loss of ambulation," said Marcio Souza, Chief Operating Officer, PTC Therapeutics, Inc. "We remain committed to work with the CHMP to clarify the open questions and are confident we will be able to demonstrate the pulmonary benefit of Translarna in non-ambulatory patients."

The clinical data supporting the extension is based on results supporting the positive impact in the Force Vital Capacity (FVC) parameters for patients treated with Translarna in study 019, a long-term, open-label study, and study 025 (STRIDE Registry) when compared to matched natural history controls. This is in addition to currently approved labeling stating that the pharmacokinetics (PK) and safety profiles are comparable between ambulatory and non-ambulatory nmDMD patients, and that no dose adjustment is necessary when patients become non-ambulatory. The CHMP opined that the comparable PK may not ensure efficacy in non-ambulatory patients since muscle mass is reduced in this patient group. PTC and members of the scientific community expressed disagreement with this understanding during the oral explanation.

Translarna received the annual renewal of its conditional marketing authorization in June 2019 for nonsense mutation Duchenne muscular dystrophy patients who are ambulatory and two years and over. In addition, in connection with the June 2019 renewal, PTC's specific obligation for the submission of the results of Study 041, an ongoing clinical trial of ataluren, has been extended to September 2022.

About ataluren (Translarna™)

Translarna (ataluren), 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, the tradename of ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged two years and older. Ataluren is an investigational new drug in the United States.

About Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy (Duchenne) is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. It 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 Duchenne can lose the ability to walk as early as age ten, followed by loss of the use of their arms. Duchenne patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties. More information regarding Duchenne is available through the Muscular Dystrophy Association and the Parent Project Muscular Dystrophy. Additionally, information and resources are available at www.duchenneandyou.com.

About PTC Therapeutics, Inc.

PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us on www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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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 contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; expansion of commercialization of Translarna and Emflaza and related regulatory submissions, including any potential revisions to the Translarna EPAR or SmPC or any potential PTC request for re-examination by the CHMP of the negative opinion with respect to expansion of the indication to include treatment of non-ambulatory patients with nmDMD; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "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: the outcome of pricing, coverage and reimbursement negotiations with third party payors for Emflaza and Translarna and any other product or product candidates that PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, which is a specific obligation to continued marketing authorization in the EEA; the eligible patient base and commercial potential of Translarna, Emflaza, or any of PTC's other products or product candidates; PTC's scientific approach and general development progress; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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 any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna and Emflaza.

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.

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