



PTC Therapeutics Announces CHMP Recommendation of Translarna™ (ataluren) Label Update for Non-Ambulatory Patients with Duchenne Muscular Dystrophy

June 29, 2020

- EU label update supports Translarna use in patients who became non-ambulatory while on therapy -

SOUTH PLAINFIELD, N.J., June 29, 2020 /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 by a majority of votes to remove the statement "efficacy has not been demonstrated in non-ambulatory patients" from the SmPC for Translarna™ (ataluren). This label change enables healthcare professionals to use their clinical judgement to make treatment decisions for their patients on Translarna who have lost ambulation.¹ The change also should support reimbursement agencies granting continued access to Translarna for patients who become non-ambulatory during the course of their treatment. The CHMP's positive opinion is subject to final approval by the European Commission, which is normally granted in a two-month time frame.

"We are excited to see that the CHMP adopted the positive opinion for this label modification allowing patients who become non-ambulatory to continue to use Translarna," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "All nonsense mutation Duchenne patients should be able to benefit from continued Translarna use, ensuring they have the best chance of preserving muscle function for as long as possible."

Translarna is the only treatment for the underlying cause of Duchenne caused by a nonsense mutation and works by restoring dystrophin production.^{1,2} It is approved by the EMA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged two years and older. Real world evidence and clinical trials have demonstrated the potential of Translarna to slow disease progression and improve outcomes before and beyond loss of ambulation:

- In a long-term, open-label extension study (Study 019), Translarna preserved lung function in non-ambulatory patients for an additional four years compared with patients treated with standard of care from a long-term natural history study (the Cooperative International Neuromuscular Research Group (CINRG) natural history database of Duchenne patients).³
- Data from the STRIDE Registry, the first international drug registry for Duchenne patients receiving Translarna, demonstrated that boys treated with Translarna and standard of care (SoC) preserved the ability to walk for years longer than those on SoC alone, as well as experienced a slower decline in lung function.⁴
- Children treated with Translarna in a real-world setting as part of the STRIDE registry were able to walk independently for an additional 3.5 years compared with a propensity-score matched cohort in the CINRG natural history study, with a median age at loss of ambulation of 14.5 years and 11 years, respectively (72% relative risk reduction).⁴
- There was a trend toward a delay in the age at decline in pulmonary function in STRIDE patients compared with CINRG patients, as measured by predicted FVC < 50% and FVC < 1 L.⁴
- These data suggest that treatment with ataluren in addition to SoC may delay loss of ambulation, as well as pulmonary functional decline, in patients with nmDMD.

References:

1. Summary of Product Characteristics. Translarna. European Medicines Agency.(More detailed recommendations for the use of Translarna will be described in the SmPC, which will be published in a revised European public assessment report (EPAR), after the final decision on this change to the marketing authorisation has been granted by the European Commission)
2. Finkel RS, et al. Phase 2a study of ataluren-mediated dystrophin production in patients with nonsense mutation Duchenne muscular dystrophy. *12, 2013, PLoS One, Vol. 8, p. e81302.*
3. McDonald CM, et al. Long-term pulmonary function outcomes in non-ambulatory patients with nonsense mutation Duchenne muscular dystrophy treated with ataluren: 240-week data from an open-label extension study. Vienna, Austria: s.n., July 6-10 2018. Poster presented at the International Congress on Neuromuscular Diseases.
4. Mercuri E, et al. Safety and effectiveness of ataluren: comparison of results from the STRIDE Registry and CINRG DMD Natural History Study. *5, 2020, Journal for Comparative Effectiveness Research, Vol. 9, pp. 341-360.*

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 all muscles, including skeletal, diaphragm, and heart muscles. Patients with Duchenne can lose the ability to walk (loss of ambulation) 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 Translarna (ataluren)

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 PTC Therapeutics

PTC is a science-driven, 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 and diversified 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 at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

For More Information:

Investors:

Alex Kane
+1 (908) 912-9643
akane@ptcbio.com

Media:

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

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; regulatory submissions and approvals, commercialization and reimbursement of Translarna; 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 Translarna and any other product or product candidates that PTC commercializes or may commercialize in the future; 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 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.

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.

 View original content: <http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-chmp-recommendation-of-translarna-ataluren-label-update-for-non-ambulatory-patients-with-duchenne-muscular-dystrophy-301084796.html>

SOURCE PTC Therapeutics, Inc.