



## CHMP Delivers Opinion on Translarna™ for Conversion to Full Authorization

September 15, 2023

- Conference call to be held at 8:45 am EDT -

SOUTH PLAINFIELD, N.J., Sept. 15, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has given a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). The negative opinion also applies to the renewal of the existing conditional authorization. PTC plans to submit a request for re-examination per EMA guidelines. Translarna will remain on the market and available to patients with nmDMD until the re-examination process is completed. Based on CHMP procedural guidance, the opinion following the re-examination process would be expected to occur in January 2024, with EC ratification of the opinion within the following 67 days.

"We are surprised and extremely disappointed by the CHMP decision, given the well-established and favorable safety and efficacy profile of Translarna," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "Of course, this decision is most devastating for the hundreds of boys and young men in Europe with nonsense mutation DMD, for whom no other approved therapies are available. We will be submitting a request for re-examination to the CHMP to reverse this opinion, as we have done previously in the regulatory history of Translarna in Europe."

Translarna received conditional marketing authorization in Europe in 2014 based on the results of Study 007, and the conditional authorization was renewed in 2017. As part of the renewal of the conditional marketing authorization, PTC agreed to a specific obligation to conduct a third placebo-controlled trial, Study 041. PTC shared the results of Study 041, which included nominally statistically significant results on several key endpoints in the overall enrolled Intent-to-Treat population of 359 boys even though it did not meet statistical significance in the primary analysis subgroup.

The data from Study 041, as well as data from the two previous placebo-controlled trials—Studies 007 and 020 — formed the basis of the CHMPs review and decision. Additional supportive evidence of efficacy included robust meta-analyses demonstrating highly statistically significant benefit on several key disease endpoints capturing different aspects of DMD, including the Six-Minute Walk Distance test (6MWD), North Star Ambulatory Assessment, and Timed Function Tests. In addition, analyses of the real-world STRIDE registry, which includes 300 boys with an average treatment duration of over 5.5 years, demonstrating that long-term Translarna therapy delays the time to loss of ambulation by 3.5 years were included as part of the data package to confirm long-term meaningful treatment benefit. In addition, Translarna has shown a favorable safety profile, with over 3,000 patients treated to date.

In the re-examination, PTC plans to focus on the clear and consistent evidence of benefit recorded in the overall population of 700 boys included in all studies (007, 020 and 041). In addition, PTC will address the decision to designate a different primary analysis subgroup in Study 041 than the subgroup previously demonstrated to be most responsive to a treatment effect on 6MWD in Studies 007 and 020. PTC will also address concerns raised regarding the robustness of the STRIDE data in supporting long-term treatment benefit.

### Today's Conference Call

PTC will hold a conference call at 8:45 am EDT today to discuss this news. To access the call by phone, please [click here](#) to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at <https://ir.ptcbio.com/events-presentations>. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

### About Translarna™ (ataluren)

Translarna (ataluren), discovered and developed by PTC Therapeutics, 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. Translarna, the tradename of ataluren, is licensed in multiple countries including Great Britain, Northern Ireland and the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged 2 years and older. Ataluren is an investigational new drug in the United States.

### About Duchenne Muscular Dystrophy (Duchenne)

Primarily affecting males, 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-20's 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 10 years old, 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 20s.

### About PTC Therapeutics, Inc.

PTC is a 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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at [www.ptcbio.com](http://www.ptcbio.com) and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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**Forward-Looking Statement**

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the commercialization of its products and product candidates; PTC's plans for interactions with the European Medicines Agency (EMA); the outcome of any re-examination process; the clinical utility and potential advantages of Translarna (ataluren); 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 PTC's products or product candidates that PTC commercializes or may commercialize in the future; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, including whether the European Medicines Agency (EMA) determines in the re-examination process that the benefit-risk balance of Translarna authorization supports renewal of such authorization or its conversion to a full approval; PTC's ability to complete 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; PTC's ability to utilize results from Study 041 to support a marketing approval for Translarna for the treatment of nmDMD in the United States and a conversion to a standard marketing authorization in the EEA; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from our trials in Translarna; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; and the factors discussed in the "Risk Factors" section of PTC's most recent 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.

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