PTC Therapeutics Announces European Commission Returns Translarna™ Opinion to CHMP For Re-evaluation

May 20, 2024

- European Commission decides against adoption of negative opinion -

- Translarna authorization remains active in Europe -

- 2024 revenue guidance paused -

WARREN, N.J., May 20, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the European Commission (EC) has decided not to adopt the CHMP's negative opinion of January 24, 2024 on the annual renewal of the conditional marketing authorization of Translarna™ (ataluren) and has returned the opinion to the CHMP for re-evaluation. As a result, Translarna remains on the market and available for patients in Europe consistent with its current marketing authorization. The EC has asked the CHMP to further consider the totality of evidence, including data from patient registries and real-world evidence, in a revised opinion.

"The maintenance of the current authorization of Translarna is a big win for boys and young men with nonsense mutation Duchenne muscular dystrophy in Europe," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "The efficacy and safety demonstrated in clinical trials and the long-term STRIDE registry support that Translarna fills an otherwise unmet need for nmDMD patients. We look forward to working collaboratively with CHMP on next steps once they are defined."

In addition, the European Medicines Agency (EMA) has informed PTC that the decision has been taken to consider the Scientific Advisory Group (SAG) meeting for Translarna held on September 5, 2023, and all the procedural steps that followed, as invalid. Furthermore, the input from the SAG meeting held in September 2023 as well as the meeting held in January 2024 will not be considered by the CHMP in any future evaluation of Translarna.

Given the inability to accurately forecast the upside impact of the continued authorization of Translarna in Europe on 2024 revenue, PTC will pause 2024 total revenue guidance at this time. Guidance will be updated at a future date.

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 for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) 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** 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 press release, 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 future interactions with the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA); the outcome of any regulatory procedures; the clinical utility and potential advantages 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 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; PTC's ability to use the clinical data from its international drug registry study and real-world evidence concerning Translarna's benefits to support a continued marketing authorization for Translarna for the treatment of nmDMD in the EEA; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from PTC's 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 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.

Acronyms:
CHMP: Committee for Medicinal Products for Human Use

nmDMD: nonsense mutation Duchenne muscular dystrophy

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