PTC Therapeutics Provides Regulatory Update on Translarna™ (ataluren) in Europe

March 28, 2025

WARREN, N.J., March 28, 2025 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the European Commission (EC) has adopted the opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) to not renew the authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy. While this action effectively removes the drug's conditional marketing authorization in the European Economic Area, the EC indicated that individual countries within the European Union can leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued use of Translarna.

"We are of course disappointed that after this prolonged period of review the European Commission has decided to adopt the CHMP negative opinion on Translarna," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "The EC's indication that European Union member states have a mechanism to maintain treatment speaks to the safety, benefit and lack of alternative therapies for boys and young men with nonsense mutation Duchenne muscular dystrophy. We look forward to working on a country-by-country basis to provide commercial drug where possible."

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 children and adults living 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. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at **www.ptcbio.com** and follow us on Facebook, X, and LinkedIn.

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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 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 expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, commercialization and other matters with respect to its products and product candidates; the clinical utility and potential advantages of Translarna (ataluren); PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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 and other regions; PTC's ability to identify other potential mechanisms by which it may provide Translarna to nmDMD patients in the EEA; whether healthcare professionals 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 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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