# PTC Announces Translarna™ Approval in Russia for the Treatment of Duchenne Muscular Dystrophy

December 4, 2020

SOUTH PLAINFIELD, N.J., Dec. 4, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Translarna™ (ataluren) has been granted marketing approval in the Russian Federation for nonsense mutation Duchenne muscular dystrophy (nmDMD). It is estimated that 13% of DMD patients have a nonsense mutation. In countries where it is approved, Translarna is currently the only medicine that targets the underlying cause of nmDMD.[1]

"Translarna was the first therapeutic ever approved for nonsense mutation Duchenne muscular dystrophy patients and we are excited to expand approval of Translarna into Russia," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, Inc. "There has been great interest from Russian physicians to be able to access a treatment that targets the underlying cause of the disease, and a number of nmDMD patients have already been identified who can potentially benefit from Translarna. Our goal is to bring this product to nmDMD patients globally and this approval marks another important milestone."

Translarna marketing approval has been granted by the Ministry of Health of the Russian Federation for the treatment of Duchenne muscular dystrophy (DMD) caused by a nonsense mutation in patients 2 years and older. Primarily affecting males, DMD is a rare, irreversible, 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.[2] It is caused by the lack of functional dystrophin protein.[2]

**About 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 is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged two years and older. Translarna is being distributed in over 50 countries. Ataluren is an investigational new drug in the United States.

**About PTC Therapeutics, Inc.**
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.

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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, including with respect to the commercialization of its products and product candidates; 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 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; 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.

[1]Birnkrant DJ, et al. Lancet Neurol. 2018;17:251–267. Kalman L, et al. J Mol Diagn. 2011;13:167–174. Bladen CL, et al. Hum Mutat. 2015;36:395–402. Pichavant C, et al. Mol Ther. 2011;19:830–840. Prior, et al. American Journal of Human Genetics 1995.

[2]About Duchenne. (n.d.). Retrieved November 30, 2020, from https://takeonduchenne.co.uk/about-duchenne/

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