STRIDE Data Show Translarna™ Delays Loss of Ambulation by More Than Five Years in Boys with Nonsense Mutation Duchenne Muscular Dystrophy

September 20, 2021

- Translarna was seen to slow disease progression compared to standard of care alone -

- Data from long-term, real-world results from 241 patients in the STRIDE* patient registry -

SOUTH PLAINFIELD, N.J., Sept. 20, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today presented real-world results from the STRIDE patient registry demonstrating that treatment with Translarna ™(ataluren) delays loss of ambulation by more than five years in boys with nonsense mutation Duchenne muscular dystrophy (nmDMD) compared to standard of care (SoC) alone. Pulmonary function decline was also delayed by 1.8 years in those treated with Translarna and SoC.

"The five-year analysis of the STRIDE registry clearly demonstrates Translarna's profound impact on changing the course of disease progression, said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We are all proud to see Translana's life-changing effect on boys with Duchenne. The results robustness showing both a five-year delay in loss of walking as well as its ability to prolong lung function confirms what we have seen in our clinical trials. These results add to the totality of the evidence of Translarna's benefit for the patients and their families."

A time-to-event analysis of five years of registry data, presented at the World Muscle Society (WMS) 2021 Virtual Congress, shows that boys treated with Translarna plus SoC had a median age of loss of ambulation of 17.9 years old compared with 12.5 years old for those on SoC alone.[1] At 12 years old, 80% of boys receiving Translarna plus SoC are still walking, compared to 52% of the boys receiving SoC alone.[1]

The median age that boys treated with Translarna reached a predicted forced vital capacity (FVC) lower than 60% was 17.6 years old, compared with 15.8 years old for those who did not receive Translarna. This delay in loss of lung function is critical, as the sub-60% threshold is considered the milestone at which patients usually start to require respiratory physical therapy.

"Watching your child losing his abilities, until he can no longer walk or even breathe without help is heartbreaking," said Filippo Buccella, patient advocate and founder of Parent Project Italy. "We are starting to see more evidence that Translarna can potentially give children many more years of freedom, and this will bring real hope to us parents."

The analysis is based on results from 241 boys in 13 countries who were enrolled in the STRIDE patient registry over the past five years.[1] The data was compared with natural history from a propensity-score matched cohort in a long-term natural history study (the Cooperative International Neuromuscular Research Group (CINRG) natural history database of Duchenne patients).[1,2]

Translarna continues to be well tolerated in nmDMD patients, and the safety results, representing 1059 patient-years of exposure, remain consistent with the therapy's known safety profile.

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 the STRIDE Registry
The STRIDE (Strategic Targeting of Registries and International Database of Excellence) Registry is an ongoing, multicenter, observational study of the safety and effectiveness of Translarna in routine care. It is the first patient data repository to provide real-world experience regarding the long-term use of Translarna in routine clinical practice. Enrolled patients will be followed for at least five years from the date of enrollment, or until withdrawal from the study.[2] As of Jan. 31, 2021, 286 patients had been enrolled across 13 countries in Europe and Israel.

STRIDE is a collaborative partnership between TREAT-NMD and PTC Therapeutics, led by a Steering Committee comprised of leading experts in Duchenne, patient advocates from around the world and PTC representatives.

The Registry also fulfills a post-marketing commitment to the Pharmacovigilance Risk Assessment Committee of the European Medicines Agency.

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 20's.

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 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. 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, on Twitter at @PTCBio, Instagram 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 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 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.

* Strategic Targeting of Registries and International Database of Excellent (STRIDE)

[1] PTC Therapeutics. Data on File.
[2] Bushby et al. First Drug Registry in Duchenne Muscular Dystrophy (DMD) to Assess Translarna™ Use, Safety, and Effectiveness in Routine Clinical Practice [Poster], Presented at the 11th European Paediatric Neurology Society Congress 2015; May 27−30, Vienna, Austria.

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