PTC Therapeutics to Present Clinical and Real-World Evidence on Translarna™ at the Annual Congress of the World Muscle Society

October 11, 2022

- Presentations include age of loss of ambulation and preservation of upper limb function following treatment with Translarna in patients with nonsense mutation Duchenne muscular dystrophy -

SOUTH PLAINFIELD, N.J., Oct. 11, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that recent clinical trial and real-world evidence on the efficacy and safety of Translarna™ (ataluren) in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) will be presented at the 27th International Annual Congress of the World Muscle Society (WMS) in Halifax, Nova Scotia, Canada.

Key presentations include:

Poster number	Title	Poster session, location and time
P19	Updated demographics and safety data from patients with nonsense mutation Duchenne muscular dystrophy receiving ataluren in the STRIDE Registry	Wednesday, Oct. 12, 2022 Poster session 1 Main poster area – Ballroom B1-B2 14:30–16:00 – DMD - Clinical
P22	Age at loss of ambulation in patients with DMD from the STRIDE Registry and the CINRG Duchenne Natural History Study: a matched cohort analysis	Wednesday, Oct. 12, 2022 Poster session 1 Main poster area – Ballroom B1-B2 14:30–16:00 – DMD - Clinical
P23	Pulmonary function in patients with Duchenne muscular dystrophy from the STRIDE Registry and CINRG Duchenne Natural History Study: a matched cohort analysis	Wednesday, Oct. 12, 2022 Poster session 1 Main poster area – Ballroom B1-B2 14:30–16:00 – DMD - Clinical
LSVP37	Safety and efficacy of ataluren in nmDMD patients from Study 041, a phase 3, randomized, double-blind, placebo-controlled trial	Friday, Oct. 14, 2022 Poster session 4 Main poster area – Ballroom B1-B2 ePoster and virtual platform only
LSVP26	Ataluren preserves upper limb function in nmDMD patients from Study 041, a phase 3 placebo-controlled trial, and the STRIDE Registry	Friday, Oct. 14, 2022 Poster session 4 Main poster area – Ballroom B1-B2 ePoster and virtual platform only

The full congress program can be found on the WMS website: https://www.wms2022.com/page/programme

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 the European Economic Area for the treatment of Duchenne in ambulatory patients aged 2 years and older. Ataluren is an investigational new drug in the United States.

About Study 041

Study 041 is the largest prospective trial conducted in Duchenne, with an Intent-to-Treat population of 359 boys with Duchenne. This international clinical trial aimed to determine the effect of ataluren on ambulation and endurance as assessed by the six-minute walk test (6MWT) in patients with Duchenne. The study also looked at other activities such as the children's ability to run and climb up and down stairs, which are crucial milestones for maintaining independence. Study 041 was designed as a 144-week, global, placebo-controlled trial, with a 72-week placebo-controlled phase, followed by a 72-week open-label extension during which all subjects would receive Translarna therapy, but remain blinded to their initial treatment

assignment.

About the STRIDE Patient 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 six years from the date of enrollment, or until withdrawal from the study.[i] As of January 2022, 307 patients had been enrolled across 14 countries.[1]

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 fulfils 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-twenties 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 age ten, 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 twenties. More information regarding Duchenne is 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 expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; PTC's expectations with respect to the licensing, regulatory submissions and 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 Brazil, Russia, the European Economic Area (EEA) and other regions, 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 complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to utilize results from 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, to support a marketing approval for Translarna for the treatment of nmDMD in the United States; 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.

[i] 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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