PTC Therapeutics Announces First Publication of Real-World Data Showing TranslarnaTM (ataluren) Significantly Preserves Ability to Walk for Longer in Children with Duchenne Muscular Dystrophy

February 3, 2020

- STRIDE registry analysis shows Translarna preserved ambulation and physical function by years compared with those in CINRG Duchenne Natural History Study, with no new safety signals1-

- Trend toward delayed worsening of pulmonary function compared with natural history study1-

SOUTH PLAINFIELD, N.J., Feb. 3, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ:PTCT) today announced real-world data showing that boys with nonsense mutation Duchenne muscular dystrophy treated with Translarna™ (ataluren) and standard of care (SoC), preserved the ability to walk for years longer than those on SoC alone. Pulmonary function was also preserved in those treated with Translarna.[1] The analysis, presented in the publication of an interim analysis of preliminary real-world data, compared children treated with Translarna in a real-world setting from the STRIDE[*] registry with a matched cohort in a long-term natural history study, CINRG[†].[1] In addition, no new safety signals were observed in the patients treated with Translarna, consistent with what has been shown in previous clinical trials.[1] The interim data have been published in the *Journal for Comparative Effectiveness Research*. Final data from the STRIDE registry is expected in 2025.

"Duchenne muscular dystrophy is a devastating disease that causes irreversible muscle wasting and progressively robs young people of their ability to walk, move, and breathe naturally without a ventilator, and it reduces their autonomy in daily life tasks," said Dr. Andrés Nascimento, Pediatric Neurology, Neuromuscular Diseases Unit, SJD Children's Hospital, Barcelona, Spain. "In a real-world setting, children and adolescents treated with Translarna experience a delay in the disease progression, are able to maintain more mobility, and have a higher level of physical autonomy concerning the course of the natural history of the disease. This is not only clinically relevant, but especially important for the quality of life of patients and their families."

Children treated with Translarna in a real-world setting as part of the STRIDE registry were able to walk independently for an additional 3.5 years compared with a propensity-score matched cohort in the CINRG natural history study, with a median age at loss of ambulation of 14.5 years and 11 years, respectively (72% relative risk reduction). [1]

Additional analyses from the registry demonstrated that Translarna sustained the ability of boys with Duchenne to complete everyday tasks by years compared with the natural history cohort. [1] In timed function tests, Translarna sustained their ability to stand up from lying down, in under 5 and 10 seconds, for three years longer than in boys treated with SoC alone.[1] Boys treated with Translarna were also still able to climb four stairs in under 5 and 10 seconds for 1.5 and 3.6 years longer, respectively, than boys on SoC alone. [1]

In addition, the analysis showed a trend toward delayed worsening of pulmonary function in routine clinical practice for patients treated with Translarna, compared to the matched patients in CINRG. Researchers evaluated FVC, a traditional measure of lung function in Duchenne patients that correlates with disease progression and mortality. [1,2] The STRIDE data showed that 32.1% of standard of care patients from the natural history cohort had an FVC of <50%, compared to only 2.2% of patients receiving Translarna.[1,2] However, the authors state that given the low number of events and the shorter duration of follow-up of patients in the STRIDE registry compared to CINRG in these interim analyses, it is premature to draw firm conclusions from these results. [1] After loss of ambulation and loss of the use of the arms, the respiratory muscles of people with Duchenne start to progressively deteriorate, leading to the risk of life-threatening respiratory complications and the need for ventilation support. [1]

"The data from the STRIDE registry are consistently confirming the benefits seen in Translarna clinical trials and the difference it is making to patients and their families – more years of being independent and physically able without reliance on a wheelchair or ventilator," said Dr. Claudio Santos, SVP, Global Medical Affairs, PTC Therapeutics.

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 5 years from the date of enrollment, or until withdrawal from the study. As of 9 July 2018, 217 patients with a mean age of 9.8 years had been enrolled across 11 countries in Europe and Israel.

Effectiveness information may include neuromuscular function (as measured for example by timed-function tests, the North Star Ambulatory Assessment, and Performance of the Upper Limb (PUL) measures, cardiac function (including echocardiogram where available), pulmonary function (including spirometry measures), and quality of life measures. Assessments of musculoskeletal health, rehabilitation, orthopedic and gastrointestinal management, as well as other measures of psychosocial management, will be collected to allow for comparison of patient health-management activities in routine clinical care to those of published treatment guidelines.

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 TREAT-NMD

TREAT-NMD is a network for the neuromuscular field that provides an infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. Since its launch in January 2007 the network's focus has been on the development of tools that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development and into the clinic, and on establishing best-practice care for neuromuscular patients worldwide. The network has developed from its European roots to become a global organization that brings together leading specialists, patient groups and industry representatives to ensure preparedness for the trials and therapies of the future while promoting best practice today.

Further information about TREAT-NMD can be found here: http://www.treat-nmd.eu/

About CINRG

The Cooperative International Neuromuscular Research Group (CINRG) Duchenne Natural History Study (DNHS; ClinicalTrials.gov identifier: NCT00468832) was a prospective, longitudinal study of more than 400 patients with Duchenne muscular dystrophy (DMD) who were followed up between 2006 and 2016 at 20 worldwide centers as part of the academic clinical trial network, CINRG.

The CINRG DNHS enrolled patients aged 2–28 years with DMD at 20 centers in nine countries from 2006 to 2016. Data from CINRG DNHS patients receiving standard of care (SoC) are used in the present analysis as a control to provide context for assessing the effects of ataluren plus SoC in patients in the STRIDE Registry. SoC refers to palliative therapies and corticosteroid treatment.

About Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy (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 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 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 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 on www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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1. Mercurio E, Muntoni F and Nascimento O *et al.* Safety and effectiveness of ataluren: comparison of results from the STRIDE Registry and CINRG DMD Natural History Study. *Journal for Comparative Effectiveness Research*. 2020. Available at:

2. Mayer OH *et al* - Characterization of Pulmonary Function in Duchenne Muscular Dystrophy. *Pulmonol.* 2015;50:487-494.

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; expansion of commercialization of Translarna and related regulatory submissions; 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; the eligible patient base and commercial potential of Translarna or any of PTC's other products or product candidates; 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.

[*] STRIDE: Strategic Targeting of Registries and International Database of Excellence

[†] CINRG: Cooperative International Neuromuscular Research Group

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