



## **PTC Therapeutics Announces New Real-World Analysis Demonstrating Translarna™ (ataluren) Slows Disease Progression in Patients with Duchenne Muscular Dystrophy**

October 4, 2019

**- Lung function data from the STRIDE Registry show a trend toward delay of decline of pulmonary function compared with those in CINRG Duchenne Natural History1 -**

SOUTH PLAINFIELD, N.J., Oct. 4, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that data from STRIDE,\* the first international registry for patients with Duchenne muscular dystrophy due to a nonsense mutation receiving Translarna (ataluren), demonstrate that Translarna preserves lung function in children and adolescents compared with a matched cohort in a long-term natural history study.<sup>1</sup> The real world analysis was presented at the 24<sup>th</sup> International Annual Congress of the World Muscle Society.

"Across ambulation, physical function and lung function, the STRIDE data demonstrate that patients receiving Translarna preserved function for years longer than patients receiving standard of care," said Stuart Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "These are real world results that provide clinicians and regulators the true picture of patient response to treatment."

Researchers evaluated FVC, a traditional measure of lung function in Duchenne patients that correlates with disease progression and mortality.<sup>2</sup> The STRIDE data showed that 32.1% of standard of care patients from the natural history cohort had a FVC of <50%, compared to only 2.2% of patients receiving Translarna after a mean total exposure of 633 days.<sup>1</sup> The data also indicates that Translarna significantly preserved patients' ability to stand up from lying and climbing stairs compared with natural history.<sup>3</sup>

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. Patients with a predicted FVC of <50% are considered to be in the late non-ambulatory stage of Duchenne.<sup>4</sup> To conduct the analysis, patients from the STRIDE Registry were matched against a comparable cohort of patients from the Cooperative International Neuromuscular Research Group Natural History Study, based on their propensity for disease progression.<sup>1</sup>

These latest data build on the STRIDE registry – time-to-event analysis, which demonstrated that the median age at which patients on Translarna lost the ability to stand up from lying in under 5 seconds (the first key clinically significant Duchenne milestone) is 12 years – 3 years later than seen with natural disease progression in untreated children [9.1 years].<sup>3</sup>

"It's very encouraging to see positive lung function results in a real world setting and provides reassurance that ataluren is slowing disease progression," said Dr. Eugenio Mercuri, Professor, Pediatric Neurology, Catholic University, and author of the study. "Respiratory failure is the primary cause of disability and death in patients with Duchenne and monitoring and preserving lung function is a key clinical priority. As the muscles progressively weaken, breathing and effective coughing gets more difficult and patients eventually become dependent on ventilation support, which is distressing for families."

The demographic characteristics of the study population from the STRIDE Registry were published in August this year in the *Journal for Comparative Effectiveness Research*.<sup>5</sup>

### **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.

Effectiveness information may include neuromuscular function (as measured 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, is to 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.

## 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 Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy 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 on the signs and symptoms of Duchenne can be found at: [www.duchenneandyou.com](http://www.duchenneandyou.com)

## About PTC Therapeutics, Inc.

PTC is a science-led, 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](http://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; PTC's expansion of commercialization of Translarna and related regulatory submissions; PTC's strategy, future operations, future financial position, future revenues, projected costs; or intended use of proceeds from its public offering of common stock and private offering of convertible senior notes; 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 Translarna; 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 nonsense mutation Duchenne muscular dystrophy (nmDMD) followed by an 18-month open-label extension, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD, and PTC's ability to perform additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; the eligible patient base and commercial potential of Translarna and PTC's other products and 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.


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

### References:

- <sup>1</sup> Tulinius M *et al.* Pulmonary function in patients with Duchenne muscular dystrophy from the STRIDE Registry and the CINRG Natural History Study: A matched cohort analysis. Abstract presented at World Muscle Society 2019, 1-5 October, Copenhagen, Denmark.
- <sup>2</sup> Mayer OH *et al.* - Characterization of Pulmonary Function in Duchenne Muscular Dystrophy *Pulmonol.* 2015;50:487-494. And Philips, M *et al.* - Changes in Spirometry Over Time as a Prognostic Marker in Patients with Duchenne Muscular Dystrophy *Am J Respir Crit Care Med* Vol 164. pp 2191-2194, 2001
- <sup>3</sup> Mercuri, E., Buccella, F. *et al.* Timed function test data in patients with Duchenne muscular dystrophy from the STRIDE Registry and the CINRG Duchenne Natural History Study: a matched cohort analysis. Poster 11-38. Presented at EPNS 2019 (16-21 September, Athens, Greece).

<sup>4</sup> Birnkrant DJ et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol* 2018;17:347–61.

<sup>5</sup> Muntoni, F., Desguerre, I. et al. Ataluren use in patients with nonsense mutation Duchenne muscular dystrophy: patient demographics and characteristics from the STRIDE Registry. *J Comp Eff Res*. 2019 Aug 15. doi: 10.2217/ce-2019-0086.

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