



PTC Therapeutics Announces Initial Data from Patient Registry Demonstrating Translarna™ (ataluren) Slows Disease Progression in Children with Duchenne Caused by a Nonsense Mutation

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- Analysis of data to date shows Translarna Preserved Ambulation for up to 5 Years Compared to Natural History -

SOUTH PLAINFIELD, N.J., Oct. 6, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced preliminary data from the first international drug registry for Duchenne patients receiving Translarna™ (ataluren), underscoring the long-term clinical benefit of Translarna when used in routine clinical practice in delaying irreversible muscle loss in children with Duchenne caused by a nonsense mutation, when compared with published natural history. The data show that children and adolescents receiving Translarna in the real-world setting are continuing to walk years longer than untreated children, and are staying more physically able. A time-to-event analysis for loss of ambulation has shown that patients on Translarna had a median age of loss of ambulation of 16.5 years of age – up to 5 years later than seen with natural disease progression in untreated children. The data were presented as a late breaker at the 23rd International Annual Congress of the World Muscle Society in Argentina.

Experience the interactive Multichannel News Release here: <https://www.multivu.com/players/English/8420051-ptc-therapeutics-stride-registry-duchenne-muscular-dystrophy-translarna/>

"The results from the Registry data are exceptionally important because they further substantiate the results observed in our analyses of data from previous clinical trials studying Translarna in Duchenne," said Stuart W. Peltz, Ph.D., CEO, PTC Therapeutics. "Delaying the loss of ambulation is critical to these patients, as it predicts the time to loss of pulmonary function and mortality."

Patients who received Translarna in routine clinical practice also experienced a slower decline in their physical function compared with the placebo arm of Phase 3 Study 020, as measured by a series of timed function tests. Safety outcomes for patients in the STRIDE Registry were consistent with the known safety profile of Translarna.

"These initial data are very encouraging because they provide the first, real-world evidence for the impact of Translarna when used routinely, longer term," said Dr. Eugenio Mercuri, Professor, Pediatric Neurology, Catholic University. "We are seeing children who have been exhibiting first clinical symptoms of Duchenne at approximately 3 years of age and yet are still able to walk years after they would normally be wheelchair-bound, which is what matters most to patients and their families."

The analysis was based on data captured from 216 patients, the majority of whom had not been previously enrolled in an ataluren clinical trial, across 11 European countries and Israel. Patients had a mean age of 9.8 years at first assessment and had been diagnosed with Duchenne caused by a nonsense mutation at approximately five years of age. Almost 90% had previously or were still receiving corticosteroids.

"Keeping a child with Duchenne walking for as long as possible is crucial not only to maintain independence, but to delay the rapid deterioration that usually follows loss of ambulation, including loss of the use of arms, which are essential for self-care, and respiratory and cardiac complications," said Filippo Buccella, an author of the study and Duchenne patient advocate. "As a father to a son with Duchenne, slowing the progression of this devastating disease is vital to maintaining quality of life for the patient as well as the family."

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. The development of ataluren has been supported by grants from the Muscular Dystrophy Association; FDA's Office of Orphan Products Development; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

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, 216 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 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 on the signs and symptoms of Duchenne can be found at: 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. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success 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.

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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; the timing and outcome of PTC's regulatory process, including with respect to Translarna; PTC's expansion of commercialization of Translarna; 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 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 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 nonsense mutation Duchenne muscular dystrophy (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 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.



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