PTC Therapeutics Announces FDA Acceptance of Translarna™ NDA Resubmission

October 30, 2024

WARREN, N.J., Oct. 30, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today the U.S. Food and Drug Administration (FDA) has accepted for review the resubmission of the New Drug Application (NDA) for Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD).

"The NDA acceptance for review is a significant milestone that brings us one step closer to providing this important treatment to boys and young men living with nonsense mutation Duchenne muscular dystrophy in the United States," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "The totality of evidence clearly supports the favorable safety profile and short- and long-term benefits of Translarna for individuals with nmDMD. We look forward to working with FDA throughout the review process."

"We thank FDA for accepting the Translarna NDA for review," stated Pat Furlong, Founder and CEO, Parent Project Muscular Dystrophy. "Translarna uniquely addresses individuals with Duchenne muscular dystrophy secondary to nonsense mutation and could provide an important treatment option for our community. Families in the United States have been waiting a long time for a treatment that targets the underlying cause of nonsense mutation Duchenne."

The NDA resubmission is based on the findings of significant benefit demonstrated in the ITT population (N=359) of the global placebo-controlled trial Study 041. Following 72-weeks of Translarna treatment, there was significant benefit demonstrated on the key study endpoints of six-minute walk distance (6MWD) (p=0.0248), NorthStar Ambulatory Assessment (p=0.0283), 10-meter walk/run (p=0.0422), 4-stair climb (p=0.0293), and time to 10% worsening of 6MWD (p=0.0078).

In addition, the NDA includes the findings of significant long-term Translarna treatment benefit as captured in the STRIDE registry. Translarna treatment resulted in a 3.5-year delay in loss of ambulation (p<0.0001) and a 1.8-year delay in reaching a predicted forced vital capacity of less than 60% (p=0.0028) a critical threshold of lung function.

"We are excited that the FDA has accepted the Translarna NDA for review," said Debra Miller, Founder and CEO of CureDuchenne. "We believe that the totality of the data demonstrates the meaningful benefits and strong safety profile of Translarna for people with Duchenne muscular dystrophy caused by a nonsense mutation, which is approximately 13% of our community. Many of our boys and young men have participated in Translarna clinical trials over the years, and about 150 of them remain on therapy through extension studies and continue to experience the benefits of Translarna, including maintaining independence."

As this was an NDA resubmission following a complete response letter to the NDA which was filed over protest in 2016, FDA is not obligated to follow PDUFA review timelines. Thus, an action date has not been provided.

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 multiple countries. Study 041 was a global placebo-controlled trial that enrolled a broad population of nmDMD patients. The study included a primary analysis population of a subgroup of enrollees, which did not reach statistical significance on the primary endpoint of 6MWD. However, significant benefit was recorded across 6MWD and other key study endpoints in the overall enrolled (ITT) population.

About the STRIDE Registry
The STRIDE 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.

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 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 20s.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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Forward-Looking Statement:
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 regulatory submissions and responses, commercialization and other matters with respect to its products and product candidates; PTC's plans for interactions with the U.S. Food and Drug Administration (FDA); the clinical utility and potential advantages of Translarna (ataluren); PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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, or PTC's ability to identify other potential mechanisms by which it may provide Translarna to nmDMD patients in the EEA; PTC's ability to use the clinical data from its international drug registry study and real-world evidence concerning Translarna's benefits to support a continued marketing authorization for Translarna for the treatment of nmDMD in the EEA; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from PTC's trials in Translarna; 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.

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