PTC Therapeutics Announces Positive Data from its Translarna™ Phase II Clinical Trial in Children as Young as Two Years with Nonsense Mutation Duchenne Muscular Dystrophy

July 9, 2018

SOUTH PLAINFIELD, N.J., July 9, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ:PTCT) today announced the presentation of data from the Translarna (ataluren) Phase II Study 030 demonstrating that the safety and pharmacokinetic profile of Translarna in children from two to five years with nonsense mutation Duchenne muscular dystrophy (nmDMD) was consistent with that for older children.[1] Importantly, the data also showed that treatment with Translarna resulted in improvements in timed function tests and the North Star Ambulatory Assessment from baseline at weeks 28 and 52, with mean changes showing as much as a 25 percent improvement after one year.[1] The data at 28 weeks formed the basis of the recent positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) to expand the current indication of Translarna to include nmDMD ambulatory children from two to five years of age.[2] The data was presented at the International Congress on Neuromuscular Diseases in Vienna.

Translarna is the only approved treatment to address the underlying cause of nmDMD, a rare, genetic, muscle-wasting disease,[1] and is currently licensed in Europe for ambulatory patients aged five years and older.[3]

"We are excited to demonstrate that Translarna showed an improvement over one year of treatment in patients with nonsense mutation Duchenne as young as two years of age," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, Inc. "Irreversible muscle damage starts before the age of five. Early intervention is critical to maintain muscle function and delay disease progression."

An interim analysis of Study 030 demonstrated that at week 28, the safety and pharmacokinetic profile for Translarna in children aged two to five years is consistent with that for older children.[1] Clinical benefits were also observed at 28 weeks with Translarna, with decreases versus baseline in the time to run/walk 10 meters, climb four stairs, and stand from lying face up (supine).[1] The most common adverse events included pyrexia, ear infection, and nasopharyngitis.[1]

Study 030 evaluated changes in timed function tests (TFTs) and the 3-part, 8-part and full (16) items North Star Ambulatory Assessment (NSAA) scales, adopted for children under five years of age (N=12).[1] Results summarized in the table below.

TFTs	**Week 28**			**Week 52**		
	Baseline (s)	**Time to complete (s) at week 28**	**Mean % change**	**Baseline (s)**	**Time to complete (s) at week 52**	**Mean % change**
Time to descend 4 stairs	7.1	6.5	7.2%	7.5	5.3	24.2%
Time to ascend 4 stairs	7.1	5.3	9.1%	7.5	4.5	23.3%
Rise from floor	7.3	4.3	17.4%	7.1	4.1	19.6%
10 m walk/run	6.7	5.9	8.4%	6.6	6.2	8.6%

NSAA	**Week 28**			**Week 52**		
Item	**Baseline (s)**	**Score at week 28**	**Mean % change**	**Baseline (s)**	**Score at week 52**	**Mean % change**
16	16.2	19.8	24.9%	16.0	21.5	36.6%
8	10.5	12.1	15.1%	10.5	12.8	23.3%
3	5.4	5.8	10.3%	5.4	5.6	6.3%

About Study 030[1]

Study 030 was an open-label, Phase 2 study designed to evaluate the safety and pharmacokinetics (PK) of ataluren (10, 10, and 20 mg/kg) in patients aged ≥2 to <5 years with nmDMD. The study includes a 4-week treatment period, a 48-week extension period, and a 4-week follow-up period. Secondary objectives in Study 030 evaluated changes in timed function tests (TFTs) and the total, 3-part,8-part and full (16) items North Star Ambulatory Assessment (NSAA) scales, adapted for children <5 years of age. All patients were male (N=14) with genotypic confirmation of nmDMD. Two patients were excluded from the current analysis: one patient did not have reported functional assessment at Week 28; and one patient did not have baseline measurement all for post-line evaluations, resulting in N=12. Seven out of the fourteen patients in the safety population (50%) reported

≥1 treatment-emergent adverse event (TEAE) during the extension phase, all of which were deemed unrelated to the study drug; there were no serious TEAEs or discontinuations due to a TEAE. Pyrexia, ear infection, and nasopharyngitis were the most common TEAEs, each occurring in 2 patients (14.3%).

About 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, tradename of ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five 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 Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy (DMD) 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 the final determination by the European Commission with respect to expanding the use of Translarna to include children aged two to five years with nmDMD and with respect to renewal of the marketing authorization in the European Economic Area (EEA) for Translarna for the treatment of nmDMD; the clinical utility and potential advantages of Translarna; PTC's ability to continue to supply Translarna to patients across Europe and in other territories; 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: 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 outcome of pricing, coverage and reimbursement negotiations with third party payors for Translarna; 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, Emflaza and PTC's other 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 or Emflaza.

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.

References

[1] Tian C, et al. Ataluren in patients aged ≥ 2 to < 5 years with nonsense mutation Duchenne muscular dystrophy (nmDMD): 28-week results from a Phase 2 study. Poster presented at the International Congress on Neuromuscular Diseases, July 6-10, 2018, Vienna, Austria. Poster 807

[2] PTC press release. CHMP Adopts Positive Opinion for the Expansion of the Translarna ™(ataluren) Label to Include Patients as Young as 2 Years of Age. Available at: http://ir.ptcbio.com/news-releases/news-release-details/chmp-adopts-positive-opinion-expansion-translarnatm-ataluren. Last accessed: June 2018.)

[3] Translarna, Summary of Product Characteristics

View original content:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-positive-data-from-its-translarna-phase-ii-clinical-trial-in-children-as-young-as-two-years-with-nonsense-mutation-duchenne-muscular-dystrophy-300677534.html

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