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Translarna™ Phase 2b Data Published in Muscle & Nerve

Data Supports Benefit in Nonsense Mutation Duchenne Muscular Dystrophy Patients

SOUTH PLAINFIELD, N.J., Oct. 27, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the publication of data and related analysis which provide strong support for concluding that Translarna™ (ataluren) was active and showed clinically meaningful improvements in Translarna-treated patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). The data was published in the October issue of *Muscle & Nerve.* Translarna received marketing authorization in the European Union in August 2014 for the treatment of ambulatory nmDMD patients five years and older.

The data demonstrated that from baseline to Week 48 nmDMD patients treated with Translarna (40 mg/kg/day given in three doses) had a 31.3 meter benefit in six minute walk distance (6MWD) relative to patients in the placebo group (post hoc p=0.056). Though having achieved the targeted treatment effect, the results did not achieve significance at the p < /=0.05 level due to a higher than expected variability in a heterogeneous patient population. In a retrospective analysis, more severely affected patients whose baseline 6MWD was less than 350 meters and were receiving Translarna had a 68.2 meter benefit relative to the placebo group (nominal p=0.0053). Patients receiving Translarna also demonstrated a slower rate of decline in ambulation, based on an analysis of time to 10 percent worsening in 6MWD. Additionally, based on a retrospective analysis, patients receiving treatment also trended better in the stair climb and stair descend time-function tests as well as other secondary endpoints of physical function. Safety results showed that Translarna was generally well tolerated. Serious adverse events were infrequent and none were considered to be related to Translarna. The most frequent adverse reactions at the recommended dose were nausea, vomiting, and headache. These adverse reactions generally did not require medical intervention, and no patients discontinued Translarna treatment due to any adverse reaction.

"Prolonging ambulatory function produces a myriad of benefits that are essential and important to boys living with DMD and affords boys a longer period of self-sufficiency," stated Professor Kate Bushby, M.D., Institute of Genetic Medicine, Newcastle University. "During a one-year clinical trial, substantial increases in muscle strength and function from a dystrophin-restoration therapy like Translarna are not generally anticipated but rather demonstrating the ability to stabilize or slow the decline in muscle function is the real goal. Translarna offers real promise for patients as a treatment that addresses the underlying cause of this rare genetic disorder."

"The results presented in this publication show that patients treated with Translarna demonstrated clinically meaningful trends across all patient groups as measured by the six minute walk distance," stated Robert Spiegel, M.D., FACP, Chief Medical Officer of PTC Therapeutics, Inc. "The results also showed that Translarna showed an even more pronounced effect for those patients who are rapidly declining in walking ability. Importantly, the results observed in the 6 minute walk test were also supported by positive trends in certain other important secondary endpoints that were measured during the course of the trial."

"We are pleased by the results observed in this study," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, Inc. "These results, as well as the work we did in defining and understanding the natural history of DMD utilizing the 6 minute walk test, give us a high degree of confidence that our ACT DMD confirmatory trial is both well designed and powered for success. We look forward to the Phase 3 trial results in 2015 and remain committed to bringing Translarna to all patients who can benefit from it."

About the Phase 2b Clinical Trial
The randomized, double-blind, placebo-controlled Phase 2b trial was designed to evaluate the safety and efficacy of 48 weeks of Translarna therapy in patients with nmDMD. The study enrolled 173 participants at 37 sites in North America, Europe, Australia, and Israel. Participants, males ≥5 years, were randomized to receive received placebo, Translarna 40 mg/kg/day or Translarna 80 mg/kg/day given at morning, midday and evening. The primary outcome measure was the total distance walked during a 6-minute walk test, a standardized test of ambulation. Other outcome measures in the study evaluated activity at home, muscle and heart function, strength, cognitive ability, muscle integrity, and muscle dystrophin expression. Safety parameters, compliance, and Translarna blood levels were also monitored.

About Translarna™ (ataluren)
Translarna, 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. PTC has received conditional marketing authorization in the European Economic Area for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Translarna is an investigational new drug in the United States. The development of Translarna has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); 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 (DMD)
Primarily affecting males, Duchenne muscular dystrophy (DMD) is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of skeletal, diaphragm, and heart muscles. Patients with DMD, the more severe form of the disorder, lose the ability to walk as early as age 10 and experience life-threatening lung and heart complications in their late teens and twenties. It is estimated that a nonsense mutation is the cause of DMD in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union. More information about DMD is available through the Muscular Dystrophy Association (www.mdausa.org), Parent Project Muscular Dystrophy (www.parentprojectmd.org), Action Duchenne (www.actionduchenne.org), United Parent Projects Muscular Dystrophy (www.uppmd.org), Muscular Dystrophy Campaign (www.muscular-dystrophy.org) and AFM (l'Association francaise contre les myopathies), (www.afm-telethon.fr).

About PTC Therapeutics, Inc.
PTC is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and in collaborations with leading biopharmaceutical companies. For more information on the company, please visit our website www.ptcbio.com.

PTC Therapeutics, Inc. Forward Looking Statements:
Any statements in this press release about future expectations, plans and prospects for PTC, the development of and potential market for PTC's product candidates, our clinical trials, including ACT DMD, our current and planned regulatory submissions, our earlier stage programs, the potential advantages of Translarna and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the timing and conduct of clinical trials, including ACT DMD, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" in PTC's most recent Quarterly Report, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent PTC's views only as of the date of this press release. You are urged to carefully consider all such factors. The forward-looking statements contained herein represent PTC's views only as of the date of this press release, and we do 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 release except as required by law.