



PTC ANNOUNCES RESULTS FROM PHASE 3 ACT DMD CLINICAL TRIAL OF TRANSLARNA™ (ATALUREN) IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY

Totality of clinical data demonstrates Translarna's ability to slow disease progression for patients with nonsense mutation Duchenne muscular dystrophy, and highlights strong safety profile

BURLINGTON, ON – Oct. 30, 2015 - PTC Therapeutics International, Ltd., a subsidiary of PTC Therapeutics, Inc., recently announced results from the Phase 3, double-blind, placebo-controlled, 48-week ACT DMD trial of Translarna™ (ataluren), an oral, first-in-class, protein restoration therapy for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). The trial results showed clinically meaningful benefits for Translarna-treated patients.

“As a clinician who focuses on this devastating disease, I am very encouraged to see a benefit of this magnitude in the one-year timeframe of the ACT DMD study,” said Dr. Craig Campbell, Head of Pediatric Neurology at London Health Sciences Centre. “DMD is a progressive, lifespan-limiting disorder, and it’s so important to have a treatment with a favorable safety profile that provides benefit for the course of the disease. This study demonstrates the clear need to treat patients quickly, and we are hopeful that Canadians with nonsense mediated DMD will be able to get timely access to this life-changing therapy leading up to and following Health Canada’s review, through the appropriate mechanisms.”

In the overall intent-to-treat study population, the primary endpoint of change from baseline in the 6-minute walk test (6MWT) demonstrated a 15 meter benefit ($p=0.213$), which was not statistically significant. A highly significant benefit of 47 metres ($p=0.007$) was demonstrated in the pre-specified patient population of 300-400 metres at baseline as measured by the 6MWT, which is in line with the company's prior experience in its Phase 2b trial and consistent with the evolving understanding of the 6MWT. Importantly, no patients in this group lost ambulation (0/47) versus four patients in the placebo group (4/52). Translarna showed a benefit over placebo across key secondary and tertiary endpoints, including timed function tests (10 meter Run/Walk, 4 Stair Climb, 4 Stair Descend) and the North Star Ambulatory Assessment test. In addition, a pre-specified meta-analysis of the combined placebo-controlled ACT DMD and Phase 2b trials demonstrated a statistically significant benefit of Translarna across the primary ($p=0.015$) and key secondary endpoints.

After the completion of ACT DMD, both placebo and treated patients were given the opportunity to continue on Translarna in an open-label extension study. Ninety-seven per cent of the patients who completed ACT DMD enrolled in the extension study.

“For boys with DMD, time is the enemy,” said Debra Chiabai, mother a child who has DMD, and Muscular Dystrophy Canada Board Member. “Over the past year, my 15-year-old son Alex has lost the ability to stand and walk. Now his arms and hands are beginning to weaken, and soon he will lose what little independence he has left. The coming weeks and months will bring changes that cannot be reversed. It is our hope that access to new treatments can improve the lives of boys with DMD.”

ACT DMD, the largest placebo-controlled study ever conducted in patients with DMD, is a multi-centre, randomized, double-blind, Phase 3 clinical trial involving 228 patients in 53 sites across 18 countries, including three sites and nine patients in Canada. Patients between the ages of seven and 16 with nmDMD were randomized to receive either Translarna 40mg/kg per day ($n=114$) or placebo ($n=114$)



over 48 weeks. The primary endpoint was change from baseline in the 6MWT. Analyses of data from pre-specified subgroups, including the pre-specified subgroup of patients with baseline 6-minute walk distance (6MWD) of 300 - 400 metres, were also completed. Key secondary outcome measures were timed-function tests, including time to run or walk 10 metres and the time to ascend or descend four stairs. Tertiary endpoints included the North Star Ambulatory Assessment test, a functional scale designed for ambulant boys affected by DMD, and the Pediatric Outcomes Data Collection Instrument (PODCI), a validated tool for measuring quality of life in pediatric patients with orthopedic conditions. Supportive analyses of ambulation were conducted, including the proportion of patients with at least 10 per cent worsening in 6MWD. A pre-specified meta-analysis of combined data from the ACT DMD and Phase 2b (ambulatory decline phase) studies was also performed.

The ACT DMD study confirmed the favorable safety profile of Translarna, which was generally well-tolerated, consistent with results from previous studies. More than 500 nmDMD patients have now received Translarna, the largest population to be treated with a disease-modifying agent in DMD.

“We are very pleased to see this promising data, which in its totality demonstrates Translarna’s ability to change the course of progression of DMD – especially at specific stages of the disease,” said Michael Cloutier, General Manager of PTC Therapeutics in Canada. “Health Canada recently initiated its review of Translarna to treat dystrophinopathy due to a nonsense mutation and we hope it will soon become available to those patients who can benefit from it.”

Translarna received marketing authorization from the European Medicines Agency (EMA) in 2014 for use in ambulatory nmDMD patients who are five years of age and older. Earlier this year, PTC filed a New Drug Submission (NDS) with Health Canada, which is currently under review. PTC intends to submit the results from the ACT DMD study to the EMA and to complete its rolling submission for a New Drug Application (NDA) to the FDA by the end of 2015.

ABOUT DUCHENNE MUSCULAR DYSTROPHY

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 per cent of patients.

ABOUT TRANSLARNA

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. Translarna is licensed in the European Economic Area 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 and Canada. The development of Translarna has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the non-profit 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.

The FDA and the European Commission have granted Translarna Orphan Drug status for the following indications: Duchenne muscular dystrophy, cystic fibrosis, Mucopolysaccharidosis I (MPS 1), and aniridia.

ABOUT PTC THERAPEUTICS

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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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, other than those of historical fact, contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the timing of PTC's planned regulatory filings, including with the FDA, the EMA and other regulatory bodies outside of the United States and European Economic Area, or EEA; PTC's ability to maintain the marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation DMD in the EEA, which is conditioned upon, among other things, completion of ACT DMD and submission of the final report, including additional efficacy and safety data from ACT DMD, during 2015 and which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; the clinical utility and potential advantages of Translarna; the rate and degree of market acceptance of Translarna; PTC's estimates regarding the potential market



opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; the timing and scope of PTC's commercial and early access program launches; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "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 final analyses of the data from ACT DMD, which may vary from PTC's initial analysis, lead to different (including more or less favorable) interpretations of the results than the analyses conducted to date, and identify further important information that is not available at the time of this press release; whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), adverse decisions by regulatory authorities, other delay or deceleration of the regulatory process, and PTC's ability to meet existing or future regulatory standards with respect to Translarna; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nonsense mutation DMD, including its ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which it may obtain regulatory approval, including the United States, EEA and other territories; the initiation, conduct and availability of data from clinical trials and studies; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna 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 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 Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nonsense mutation Duchenne muscular dystrophy.

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 release except as required by law.