



May 23, 2014

## **PTC THERAPEUTICS RECEIVES POSITIVE OPINION FROM CHMP FOR TRANSLARNA™ (ATALUREN)**

### **- The first treatment for the underlying cause of Duchenne muscular dystrophy -**

SOUTH PLAINFIELD, NJ – May 23, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that following its request for re-examination, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion regarding the company's application for a conditional marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged five years and older.

"We are very pleased with the outcome of the CHMP review of Translarna's marketing authorization application (MAA) and the level of engagement we experienced with CHMP members throughout the review process," said Robert J. Spiegel, M.D., Chief Medical Officer of PTC Therapeutics, Inc. "We are grateful to the patients, families, advocacy groups and physicians who have supported PTC Therapeutics through many years of research and development of Translarna. It is important to note that this journey continues through the completion of our Phase 3 Translarna confirmatory trial in nmDMD (ACT DMD) which is a high priority for PTC and the DMD community."

Dr. Craig McDonald, Professor of Physical Medicine and Rehabilitation at the University of California, Davis, who developed and validated the 6-minute walk test as a primary clinical endpoint in Duchenne muscular dystrophy (DMD) stated, "This is a historic day for the DMD community. Translarna is the first treatment for the underlying cause of nonsense mutation DMD to receive a positive opinion from the CHMP. The Phase 2b clinical trial provided strong evidence that Translarna slows disease progression as measured by the 6-minute walk test. A clinically meaningful 31.3 meter benefit in 6-minute walk distance, relative to placebo, was achieved in 48 weeks of treatment in patients five years and older and this was supported by positive trends in multiple secondary efficacy endpoints. In addition, in the prespecified group of patients with less than a 350 meter baseline 6-minute walk distance, a 68 meter benefit was observed in patients treated with 40 mg/kg Translarna given daily, relative to placebo. A conditional approval by the European Commission based on this positive opinion would allow children with nmDMD in the European Union to gain access to Translarna while PTC Therapeutics completes its ongoing confirmatory trial."

The CHMP opinion will form the basis for a European Commission (EC) decision as to whether to formally grant the conditional marketing authorization. The European Commission will review the positive opinion from the CHMP and generally delivers its final decision within three months. The conditional marketing authorization would authorize the company to market Translarna with unified labeling in the 28 countries that are Member States of the European Union, as well as European Economic Area members Iceland, Liechtenstein and Norway.

"We congratulate PTC Therapeutics on this landmark decision by the CHMP," stated Elizabeth Vroom, Chair of United Parent Project Muscular Dystrophy (UPPMD). "We applaud PTC for its dedication to the community and its perseverance in pursuing regulatory options to provide Translarna to patients as rapidly as possible. The company's pioneering work has paved the way and encouraged the scientific community to develop new therapies for DMD," she continued. "The EMA is to be commended for recognizing the great unmet need for novel treatments for this relentlessly progressive disease."

Filippo Buccella, President of Parent Project Italy and UPPMD board member, commented, "After thirty years since the discovery of the dystrophin gene, we are finally beginning to see a change in the landscape. For the first time in the history of Duchenne, we see the path to approval for a drug to treat the underlying cause of DMD. This positive result rewards the efforts of a company that has always believed in collaboration with patients and with clinicians. The perseverance of this community made it possible to realize a dream that can give hope to the boys affected with nmDMD in Europe. Our work as a community of patients is not yet finished and we will stand ready to participate with PTC in the ensuing stages of this process and, we hope, with many other companies with new therapies for Duchenne."

PTC requested a re-examination of the CHMP's negative opinion received in January 2014. The positive opinion is based on data and subsequent analysis submitted from a 48-week, 174-patient Phase 2b double-blind, placebo controlled trial which demonstrated that nmDMD patients treated with Translarna (40 mg/kg given daily) walked on average 31.3 meters farther than patients on placebo, as measured by the change in six-minute walk distance (6MWD) from baseline to Week 48. Patients receiving Translarna also demonstrated a slower rate of decline in ambulation, based on an analysis of time to 10 percent worsening in 6MWD. Safety results showed that Translarna was generally well tolerated. Serious adverse events were infrequent and none were considered to be related to Translarna. PTC's global Phase 3 ACT DMD clinical trial is ongoing with

full enrollment expected mid-2014.

"The positive opinion from the CHMP recommending the conditional approval of Translarna in nonsense mutation Duchenne muscular dystrophy is a major milestone for the DMD community and we are extremely proud of this joint achievement in accelerating the access to Translarna for patients with nonsense mutation DMD," stated Stuart W. Peltz, Ph.D., CEO of PTC Therapeutics, Inc. "DMD is a progressive disease for which there are currently no approved treatment options. As previously disclosed, we expect to have all patients enrolled in our global Phase 3 ACT DMD by mid-2014. The outcome of this trial is critical for achieving full approval in the EU as well as the US. Assuming that the EC approves a conditional marketing authorization for Translarna in nmDMD, today's decision means that in parallel to this effort, we will be able to provide patients access to Translarna with the immediacy that DMD deserves."

#### ABOUT CONDITIONAL APPROVAL

Conditional approval is granted based on a positive benefit/risk ratio in the available data which, while not yet comprehensive, indicate that the public health benefits of immediate availability of a medicine outweigh its risks. The company is given obligations to fulfill by the EC, such as the performance of further studies. The approval is renewed on a yearly basis until all obligations have been fulfilled, and is then converted from a conditional approval into a full approval. Conditional approvals can only be granted for medicines that satisfy an unmet medical need, meaning the medicine is intended to be used for a disease or condition for which no treatment is readily available, and it is therefore important that patients have early access to the medicine concerned.

#### 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. 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](http://www.mdausa.org)), Parent Project Muscular Dystrophy ([www.parentprojectmd.org](http://www.parentprojectmd.org)), Action Duchenne ([www.actionduchenne.org](http://www.actionduchenne.org)), United Parent Projects Muscular Dystrophy ([upmd.org](http://upmd.org)), Muscular Dystrophy Campaign ([www.muscular-dystrophy.org](http://www.muscular-dystrophy.org)) and AFM (l'Association française contre les myopathies), ([www.afm-telethon.fr](http://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](http://www.ptcbio.com).

#### FOR MORE INFORMATION PLEASE CONTACT

Jane Baj  
+1 (908) 912-9167  
[jbaj@ptcbio.com](mailto:jbaj@ptcbio.com)

#### FORWARD LOOKING STATEMENTS:

All statements, other than those of historical fact, contained in this press release, including statements regarding the future expectations, plans and prospects for PTC, the timing of regulatory approvals, including any determination (whether positive or negative) by the European Commission with respect to conditional marketing authorization for Translarna in nmDMD, the development of and potential market for Translarna, including our estimates regarding the size of the nmDMD patient population, our Phase 3 clinical trial for Translarna in nmDMD, including the timing of enrollment for such trial, our ability to satisfy the obligations necessary to obtain full approval for Translarna in nmDMD, and the objectives of management, are forward-looking statements. Other forward-looking statements may be identified by the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

Our actual results, performance or achievements could differ materially from those expressed or implied by forward-looking

statements we make as a result of a variety of risks and uncertainties, including among others, those related to our expectations for regulatory approvals, including the European Commission's determination with respect to conditional marketing authorization for Translarna in nmDMD, the initiation and conduct of clinical trials, availability of data from clinical trials, our scientific approach and general development progress, the availability or commercial potential of our product candidates, market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, and the other factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission. You are urged to carefully consider all such factors. In addition, the forward-looking statements included in this press release represent our views only as of the date of this release. We anticipate that subsequent events and developments will cause our views to change. However, while PTC may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so. These forward-looking statements should not be relied upon as representing PTC's views as of any date subsequent to the date of this press release.