



January 24, 2014

## **PTC THERAPEUTICS PROVIDES UPDATE ON CHMP OPINION FOR CONDITIONAL APPROVAL OF ATALUREN FOR NONSENSE MUTATION DUCHENNE MUSCULAR DYSTROPHY**

SOUTH PLAINFIELD, NJ – January 24, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) (PTC) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a negative opinion on the Company's marketing authorization application (MAA) for conditional approval of ataluren for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). The response from the CHMP is consistent with the Company's previous guidance concerning the substantial risks regarding conditional EMA approval and underscores the importance of PTC's work in completing the ongoing confirmatory Phase 3 clinical trial. In April 2013, PTC initiated a 48-week, 220-patient confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD. The trial is on track to complete enrollment in mid-2014 with top-line data expected in mid-2015. PTC intends to request a re-examination of the CHMP opinion with a final outcome expected in the second quarter of 2014, when the confirmatory study is expected to be more fully enrolled.

"We believe our meetings with the CHMP and its Scientific Advisory Group (SAG) were very productive and provided an opportunity for both PTC and independent DMD experts to discuss and explain key issues relating to DMD," stated Robert J. Spiegel, M.D., Chief Medical Officer of PTC. "We understand that, at this time, the potential impact of a conditional approval on the enrollment of the ongoing confirmatory Phase 3 trial is a major concern for the CHMP. We are committed to completing the trial and to bringing ataluren to nmDMD patients. We will continue to work closely with the EMA through the re-examination process in order to bring ataluren to patients as soon as possible."

Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC, added, "While we appreciate that this delay is a disappointment for families living with this disorder, we are encouraged by the detailed discussions we have had with the EMA through this application process. We understood and communicated that there was a substantial risk that the EMA would not grant us conditional approval when we began this process 15 months ago. However, we pursued this approach because we believe ataluren has shown a clinically meaningful benefit for nmDMD patients in our trials, has been generally well tolerated and should be made available to patients as soon as possible. Furthermore, we have made meaningful progress both in validating the 6-minute walk distance as an outcome measure in DMD clinical studies and in sharing an understanding of the natural history of this disorder as it relates to changes in ambulation."

### **ABOUT THE EMA RE-EXAMINATION PROCESS**

In the event that a negative opinion is adopted by the CHMP, an applicant for an MAA may request a re-examination of the opinion. Within 15 days of receipt of the opinion, the applicant must notify the EMA in writing of the applicant's intent to request a re-examination. Within 60 days of receipt of the negative opinion, the applicant must submit a document explaining the basis for its request for re-examination. The CHMP has 60 calendar days to consider the applicant's request for re-examination. If an appeal is unsuccessful, the Company could resubmit a new application in the EU at a later date.

### **ABOUT ATALUREN**

Ataluren, an investigational new drug 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 nmDMD. The development of ataluren 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 Duchenne muscular dystrophy, 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. There are an estimated 35,000 patients with DMD in the United States and Europe, and approximately 13 percent of all DMD cases are caused by nonsense mutations in the dystrophin gene. 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 ([uppm.org](http://uppm.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 Therapeutics (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 form the basis for 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:

Any statements in this press release about future expectations, plans and prospects for the Company, the development of and potential market for the Company's product candidates, the Company's Phase 3 clinical trials for ataluren in nmDMD, the Company's current and planned filings with regulatory authorities, including the Company's intent to request a re-examination of the CHMP opinion and the timing of the final outcome of such re-examination, 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 initiation and conduct of clinical trials, 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 the 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 the Company's views only as of the date of this release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.