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PTC Completes Rolling NDA Submission to FDA and Submits Phase 3 ACT DMD Clinical Trial Results to EMA for Translarna™ (ataluren) for Treatment of Nonsense Mutation Duchenne Muscular Dystrophy

SOUTH PLAINFIELD, N.J., Jan. 8, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the completion of its rolling submission of a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for Translarna™ (ataluren), an oral, first-in-class, protein restoration therapy for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). PTC has also fulfilled the principal requirement of the European Medicines Agency (EMA) in connection with its approval of Translarna in August 2014 by submitting the results of its Phase 3 ACT DMD clinical trial to the agency.

"The completion of these regulatory submissions for Translarna is a significant milestone for PTC," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Following Translarna's approval within Europe and other countries as the first-ever treatment for an underlying cause of Duchenne muscular dystrophy, we are planning to bring Translarna to patients in the United States. We believe the results across multiple clinical studies demonstrate Translarna's ability to modify disease progression in nonsense mutation DMD and confirm its strong safety profile. We appreciate the dedication and support of the clinical investigators, patients and their families, and we look forward to working with regulators to continue to bring this much needed treatment to patients."

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 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 nonsense mutations account for approximately 13% of DMD cases, or approximately 2,000 patients in the United States.

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. 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. The development of Translarna has been supported by grants from the 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.

Translarna has the potential to benefit patients with genetic disorders caused by a nonsense mutation. On average, 11% of every monogenic disorder is caused by a nonsense mutation. PTC's strategy is to expand the clinical development of Translarna across multiple genetic disorders to deliver on the company's commitment to address rare and neglected disorders.

The FDA and the EMA have granted Translarna Orphan Drug status for the following indications: DMD, cystic fibrosis (CF), Mucopolysaccharidosis I (MPS I), 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 regulatory process, including as it relates to our submissions with the FDA, the EMA and other regulatory bodies outside of the United States and European Economic Area, or EEA and related regulatory reviews; the clinical utility and potential advantages of Translarna; the timing and scope of PTC's commercial and early access program launches; 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; 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: whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD and other data with respect to the safety and efficacy of Translarna for the treatment of nmDMD; 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 maintain the marketing authorization of Translarna™ (ataluren) for the treatment of nmDMD in the EEA, which is subject to certain conditions and is also subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, 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; expectations for regulatory approvals; 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 nmDMD.

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.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/ptc-completes-rolling-nda-submission-to-fda-and-submits-phase-3-act-dmd-clinical-trial-results-to-ema-for-translarna-ataluren-for-treatment-of-nonsense-mutation-duchenne-muscular-dystrophy-300201465.html>

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