March 6, 2017

PTC Therapeutics Announces FDA Acknowledgment of New Drug Application Filing for Translarna™ for the Treatment of Nonsense Mutation Duchenne Muscular Dystrophy

- FDA has assigned a PDUFA date of October 24, 2017 -

SOUTH PLAINFIELD, N.J., March 6, 2017 /PRNewswire/**--** PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the U.S. Food and Drug Administration (FDA) has acknowledged the filing over protest of PTC's New Drug Application (NDA) for Translarna™ (ataluren), an oral, first-in-class, protein restoration therapy for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). The Company is seeking approval to market the drug for the treatment of nmDMD patients in the United States. Translarna received marketing authorization for patients with nmDMD in the European Union in August 2014 and is now available in over 25 countries.

The FDA has granted standard review and assigned a Prescription Drug User Fee Act (PDUFA) date of October 24, 2017. The PDUFA date is the target date for the FDA to complete its review of the NDA.

"We look forward to working closely with the FDA and the DMD community to bring this much-needed therapy to patients," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We believe that the totality of clinical data in our NDA, which includes the results of two of the largest placebo-controlled DMD clinical trials ever conducted, demonstrates Translarna's benefits to patients and merits a full and fair review by the FDA, including an advisory committee meeting."

Primarily affecting males, Duchenne muscular dystrophy 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 in their early teens 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.

PTC used the FDA's file over protest regulations to file the NDA. These regulations allow a company to have its NDA filed and reviewed following receipt of a refuse to file determination.

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 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 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 and oncology. 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 PDUFA date for the NDA; PTC's plans to work closely with the FDA during the regulatory review process; the clinical utility and potential advantages of Translarna (ataluren); whether an advisory committee meeting will be granted by the FDA; 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 "look forward", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "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: PTC's ability to resolve the matters set forth in the Refuse to File letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD, including whether PTC's filing of the NDA over protest with the FDA will result in a timely or successful review of the NDA, and whether PTC will be required to perform additional clinical and non-clinical trials or analyses at significant cost, which, if successful, could potentially support the approval of the NDA filed over protest or a new NDA submission; whether an advisory committee (if any) is convened in connection with the NDA process, and if convened, the recommendation such committee provides to the FDA for Translarna for the treatment of nmDMD; delays in PTC's projected regulatory timeline for the NDA; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open label extension; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna for the treatment of nmDMD; PTC's scientific approach and general development progress; the outcome of ongoing or future clinical studies in Translarna and PTC's other product candidates; expectations for regulatory approvals; PTC's ability to meet existing or future regulatory standards with respect to Translarna; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; 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 for Translarna for the treatment of nmDMD 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 press release except as required by law.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-fda-acknowledgment-of-new-drug-application-filing-for-translarna-for-the-treatment-of-nonsense-mutation-duchenne-muscular-dystrophy-300418256.html

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