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PTC Therapeutics Receives Complete Response Letter for Ataluren's NDA

SOUTH PLAINFIELD, N.J., Oct. 25, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the Office of Drug Evaluation I of the U.S. Food and Drug Administration (FDA) has issued a complete response letter (CRL) for the New Drug Application (NDA) of the investigational medicine ataluren for the treatment of nonsense mutation dystrophinopathies.

"We are extremely disappointed for the Duchenne community and strongly disagree with the agency's conclusions," said Stuart W. Peltz, Ph.D., chief executive officer of PTC Therapeutics. "We believe that this decision fails to consider the benefit-risk of ataluren and the high unmet medical need. Therefore, we plan to file a formal dispute resolution request next week."

The letter from the Office of Drug Evaluation I of the FDA stated that it is unable to approve the application in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. The letter also mentioned other nonclinical and CMC matters that PTC is in the process of addressing.

About ataluren (Translarna™)
Ataluren, 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, tradename of ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Ataluren is an investigational new drug in the United States.

About Duchenne Muscular Dystrophy
Primarily affecting males, Duchenne muscular dystrophy (DMD) is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. It 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 can lose the ability to walk as early as age ten, followed by loss of the use of their arms. DMD patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties. It is estimated that a nonsense mutation is the cause of DMD in approximately 13 percent of patients.

About PTC Therapeutics
PTC is a global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using our expertise in RNA biology. 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. Since its founding nearly 20 years ago, PTC's mission has focused on developing treatments to fundamentally change the lives of patients living with rare genetic disorders. The company was founded in 1998 and is headquartered in South Plainfield, New Jersey. 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 contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; PTC's plans for further interactions with the FDA; the outcome of any formal dispute resolution request; the clinical utility and potential advantages of Translarna (ataluren); 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 PTC's ability to resolve the matters set forth in the Complete Response letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD either via outcome of any formal dispute resolution request or other interactions with the FDA, and PTC's ability to perform additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; 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; the outcome of ongoing or future clinical studies in Translarna; expectations for regulatory approvals; PTC's ability to meet existing or future regulatory standards with respect to Translarna; 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.

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.

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