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# PTC Therapeutics Receives Formal Dispute Resolution Request Decision from the FDA's Office of New Drugs

SOUTH PLAINFIELD, N.J., Feb. 20, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the Office of New Drugs of the U.S. Food and Drug Administration has reiterated the FDA's prior position and denied PTC's appeal of the Complete Response Letter in relation to the New Drug Application (NDA) for ataluren. In its letter, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nonsense mutation Duchenne muscular dystrophy (nmDMD) patients' muscles, as quantified by procedures to be agreed upon between PTC and the FDA and using newer technologies. The letter adds that PTC's Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework. In a clarification teleconference with the FDA promptly after receiving the letter, PTC indicated its intent to follow the FDA's recommendation and preliminarily discussed methods to collect such dystrophin data and expedite this potential path forward.

Based on these interactions, PTC currently intends to maintain patients in the U.S. currently receiving ataluren for nmDMD through an expanded access clinical program during this process.

**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 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; the timing of and likelihood of success of its regulatory path forward, including as it relates to 1) any clinical trials and non-clinical studies to generate data on dystrophin production in ataluren, 2) a re-submission of an NDA for ataluren to the FDA, and 3) any further interactions between PTC and the FDA; 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, 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 and the FDA the results of clinical studies, including 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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