PTC Therapeutics Announces Publication of Data Demonstrating the Clinical Differentiated Benefit of Deflazacort

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- Post hoc analysis of the placebo arm of ACT DMD Data Published in Muscle & Nerve -

SOUTH PLAINFIELD, N.J., July 24, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the publication of data in *Muscle & Nerve* comparing the efficacy and safety of deflazacort and prednisone/prednisolone from the placebo arm of the ACT DMD study. The results demonstrated a clinically differentiated benefit of deflazacort over prednisone/prednisolone in slowing disease progression as measured using physical function endpoints and the time to delay loss of ambulation. Duchenne muscular dystrophy patients treated with deflazacort had notably less decline from baseline in 6-minute walk distance at Week 48 than those treated with prednisone/prednisolone. The extrapolated time to loss of ambulation was 8.58 years for deflazacort and 4.74 years with prednisone/prednisolone.

"This publication supports the benefit of deflazacort in slowing the progression of Duchenne compared to other corticosteroids," said Stuart W. Peltz, Ph.D. Chief Executive Officer of PTC Therapeutics, Inc. "The data indicates that deflazacort should be the standard of care for all patients with Duchenne. The availability of deflazacort, a treatment that has the potential to alter the natural history of Duchenne, supports the need for early diagnosis in patients with this disease."

Study results measuring other clinical endpoints monitoring physical function in Duchenne patients are also consistent with deflazacort having a differentiated clinical benefit over prednisone/prednisolone. The time function tests, such as the 4-stair climb, 4-stair descend, rise from supine, 10-m walk/run, and the North Star Ambulatory Assessment total score notably favored deflazacort over prednisone/prednisolone. Furthermore, the safety profiles for deflazacort and prednisone/prednisolone were generally comparable.

ACT DMD Study Design
The Ataluren Confirmatory Trial in patients with nonsense mutation Duchenne muscular dystrophy (ACT DMD, NCT01826487) study was a randomized, double-blind, placebo-controlled 48-week, phase 3 trial that evaluated ataluren's treatment effect in stabilizing motor function and delaying disease progression in patients with nonsense mutation Duchenne. All patients enrolled in this study had been receiving corticosteroid therapy (deflazacort or prednisone/prednisolone) for ≥6 months at study entry, had no clinically significant change in dosage or dosing regimen for ≥3 months before study entry, and were expected to maintain a stable dose and regimen during the study. The placebo arm consisted of 114 patients, 53 of whom received deflazacort and 61 of whom received prednisone/prednisolone at entry and throughout the study. The study enrolled ambulatory male patients with phenotypic and genotypic confirmation of nonsense mutation Duchenne aged 7-16 years.

About Duchenne Muscular Dystrophy
Primarily affecting males, Duchenne muscular dystrophy (Duchenne) 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, 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. More information about Duchenne is available through the Muscular Dystrophy Association (www.mdausa.org), Parent Project Muscular Dystrophy (www.parentprojectmd.org), Action Duchenne (www.actionduchenne.org), United Parent Projects Muscular Dystrophy (uppmd.org), Muscular Dystrophy Campaign (www.muscular-dystrophy.org) and AFM (l'Association francaise contre les myopathies), (www.afm-telethon.fr).

About PTC Therapeutics
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward Looking Statements:
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 clinical utility and potential advantages of Emflaza™ (deflazacort); 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: the outcome of pricing, coverage and reimbursement negotiations with third

party payors for Emflaza and Translarna; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to realize the anticipated benefits of the acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; the eligible patient base and commercial potential of Translarna, Emflaza and PTC's other product candidates; statements related to our expectations with respect to the closing of our planned acquisition of Agilis Biotherapeutics, Inc., and the other transactions contemplated in conjunction with the acquisition, including with respect to matters of timing, the anticipated financial impact and potential benefits to us, and related integration matters; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K 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 any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or Emflaza.

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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