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New Analysis of ACT DMD Placebo Data on Corticosteroids Presented at International Society for Pharmacoeconomics and Outcomes Research 22nd Annual International Meeting

- Analysis includes data from ACT DMD on deflazacort and prednisone/prednisolone -

SOUTH PLAINFIELD, N.J., May 23, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the presentation of new post-hoc analyses from the placebo arm of its ACT DMD Phase 3 trial with respect to the effect of corticosteroids on multiple measures of disease progression. Siva Narayanan, Vice President and Global Head, Market Access, Global Value and Health Economics Division, will present at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) 22nd Annual International Meeting. The scientific innovation presentation, "Disease Burden and Treatment Landscape in Duchenne Muscular Dystrophy (DMD) in the United States" will be held today at session 3 of the research poster presentations at the Sheraton Boston Hotel and the John B. Hynes Veterans Memorial Convention Center in Boston.

"The new approved treatments available to Duchenne muscular dystrophy patients have the potential to slow the progression of this devastating disorder," said Siva Narayanan, Vice President and Global Head, Market Access, Global Value and HE Division at PTC Therapeutics. "Patients taking deflazacort in the placebo arm of the ACT DMD study showed a benefit in muscle function which allows us to estimate a corresponding delay of time to loss of ambulation¹. Studies have shown that delaying loss of ambulation correlates with a delay in critical other functions. Slowing the progression of this debilitating disease could improve the lives of patients and their families^{2,3}."

For more information, visit <https://www.ispor.org/ScientificPresentationsDatabase/Presentation/71977?pdfid=49894>.

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 please visit www.ptcbio.com.

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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 deflazacort; the timing, results and conduct of PTC's clinical trials and studies of deflazacort for the treatment of DMD; 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 "guidance", "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 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.

1. Treat-NMD. http://www.treat-nmd.eu/downloads/file/meetings/2013/workshop/Session1/McDonald_NH.pdf. [Accessed May 10, 2017]
2. Ryder et al. Orphanet J Rare Dis. 2017; 12(1):79.
3. Wei Y et al. J of Neuromuscul Dis. 2015;2(3):313-324.

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