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PTC Therapeutics Announces Publication of Data from ACT DMD in The Lancet

SOUTH PLAINFIELD, N.J., July 18, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the publication of the results of the ACT DMD trial study ataluren, the only treatment for the underlying cause of nonsense mutation Duchene muscular dystrophy (nmDMD), in the current issue of *The Lancet.*

"There is a critical need for disease-modifying therapies for this devastating fatal disorder," stated Craig M. McDonald, M.D., lead author and an investigator of ACT DMD and professor of Pediatrics and chair of the Department of Physical Medicine and Rehabilitation at the University of California Davis, School of Medicine. "The data in the publication show that ataluren provides benefit for nonsense mutation Duchenne patients who were in the functional range where a treatment benefit can be seen in a one-year trial. The slowing or stabilizing of disease progression and motor function is a highly valuable effect of drug treatment which likely translates to longer-term benefits."

ACT DMD is a multi-center, randomized, double-blind, Phase 3 clinical trial involving 228 patients in 53 sites across 18 countries. Patients with nmDMD were randomized to receive either ataluren 40mg/kg per day (n=114) or placebo (n=114) over 48 weeks. The primary endpoint was change from baseline in the six-minute walk test. Treatment effects are more likely to be observed in patients in the transition stage of disease (baseline six-minute walk distance of 300-400 meters). Analyses of data from pre-specified subgroups, including the pre-specified subgroup of patients with baseline six-minute walk distance of 300 - 400 meters, were also completed. Key secondary outcome measures were timed-function tests, including time to run or walk 10 meters and the time to ascend or descend four stairs. Exploratory efficacy endpoints were change in physical function as assessed by change in the North Star Ambulatory Assessment, parent-reported health related quality of life, and activities of daily living.

"We are proud to have the results of the ACT DMD study published in *The Lancet* and highlighted in a commentary," said Stuart W. Peltz, Ph.D., chief executive officer, PTC Therapeutics, Inc.

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 its corporate headquarters are located in South Plainfield, New Jersey. For more information on the company, please visit our website www.ptcbio.com.

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 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. The development of ataluren 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.

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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 Translarna™ (ataluren); 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 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; the recommendation the advisory 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; 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.

View original content:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-publication-of-data-from-act-dmd-in-the-lancet-300489694.html

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