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New Research Validates Translarna's™ (ataluren) Mechanism of Action to Promote Readthrough of Nonsense Mutations and Produce Full-length Functional Protein

- Data published in the Proceedings of National Academy of Sciences (PNAS) -
- Results show ataluren targets the protein production machinery -

SOUTH PLAINFIELD, N.J., Oct. 4, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the *Proceedings of the National Academy of Sciences* (PNAS) has published new results further validating Translarna's™ (ataluren) mechanism of action to promote readthrough of premature stop codons resulting from nonsense mutations in genetic disorders. The results reported in PNAS establish ataluren's ability to alter the protein production process at premature stop codons and to promote the insertion of specific amino acids and restore the production of a full-length functional protein.

"These new results help us better understand ataluren's mechanism of action as well as confirm its protein restoration effect in genetic disorders," said Stuart W. Peltz, Ph.D., co-founder and Chief Executive Officer of PTC Therapeutics. "These results further support our clinical findings demonstrating the production of full-length functional protein in nonsense mutation Duchenne muscular dystrophy and cystic fibrosis. Given this mechanism, ataluren offers the potential for a new therapeutic approach for multiple nonsense mutation genetic disorders by targeting the underlying cause of the disease."

The results published by PTC Therapeutics, Dr. Allan Jacobson and his team at the University of Massachusetts Medical School, and Dr. David Bedwell and his team at the University of Alabama, demonstrate ataluren treatment produces a protein that is similar to the protein from cells that do not have a nonsense mutation. The findings were verified in multiple nonsense mutation models. In addition, there have been almost 40 publications to date, many by independent investigators, demonstrating the clinical activity of Translarna across a spectrum of rare diseases.

"These data provide new insight on ataluren's effect on protein production and validates that it targets the source of nonsense mutation genetic disorders," said Allan Jacobson, Ph.D., co-founder and Board member of PTC Therapeutics and the Gerald L. Haidak, MD and Zelda S. Haidak professor of cell biology and chair of microbiology and physiological systems at University of Massachusetts Medical School. "Therapeutic nonsense suppression is a potentially powerful approach for the treatment of the large number of genetic disorders caused by nonsense mutations."

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

About PTC Therapeutics
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. 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. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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Forward Looking Statements:

All statements, other than those of historical fact, contained in this press 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 "potential," "plan," "target," "anticipate," "believe," "estimate," "expect," "intend," "may," "project," "possible," "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: PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines that the benefit-risk balance of Translarna supports renewal of PTC's marketing authorization in the EEA; the nature and scope of any new nonsense mutation Duchenne muscular dystrophy (nmDMD) trial that PTC may design with the input of the EMA and PTC's ability to enroll, fund and conduct such trial; the outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including whether PTC is required to perform additional clinical and non-clinical trials at significant cost and whether such trials, if successful, may enable FDA review of a New Drug Application submission by PTC; the EMA's determinations with respect to PTC's variation submission which seeks to add Translarna for the treatment of nonsense mutation cystic fibrosis to PTC's marketing authorization in the EEA; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; the outcome of ongoing or future clinical trials or studies, including ACT CF and the Phase 2 study of Translarna for nmDMD in pediatric patients; 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, including its ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), the period during which the outcome of regulatory reviews will become available, adverse decisions by regulatory authorities, other delay or deceleration of the regulatory process, and PTC's ability to meet existing or future regulatory standards with respect to Translarna; PTC's ability to fulfill any additional obligations, including with respect to further trials or studies relating to cost-effectiveness, obtaining licenses or satisfying requirements for labor and business practices, in the territories in which it may obtain regulatory approval, including the United States, EEA and other territories; the initiation, conduct and availability of data from clinical trials and studies; PTC's scientific approach and general development progress; the sufficiency of PTC's cash resources and PTC's ability to obtain adequate financing in the future for PTC's foreseeable and unforeseeable operating expenses and capital expenditures; 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.

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 Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/new-research-validates-translarnas-ataluren-mechanism-of-action-to-promote-readthrough-of-nonsense-mutations-and-produce-full-length-functional-protein-300338738.html

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