

PTC Therapeutics Appoints Dr. Tuyen Ong as Chief Medical Officer

SOUTH PLAINFIELD, N.J., April 26, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Tuyen Ong, M.D. has been promoted to Chief Medical Officer. Dr. Ong joined PTC in 2014 as the Senior Vice President, Head of Clinical Development and Translational Research. He succeeds Robert J. Spiegel M.D., FACP, who has served as PTC's Chief Medical Officer since 2011. Dr. Spiegel will remain active in a consulting role, continuing to support PTC's development programs and its global clinical and regulatory activities.

"Tuyen has been a valuable addition to the PTC team, bringing a wealth of industry experience and drug development expertise," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "He will continue to play an important role in the development of Translarna for rare genetic disorders as well as the advancement of our company pipeline. I thank Bob for his significant contributions to PTC over the last five years. He has worked closely with Tuyen and the entire clinical team in advance of this transition to lay the groundwork to support our future clinical and regulatory milestones."

Prior to joining PTC in 2014, Dr. Ong served as Vice President of Global Clinical Development and Operations at Valeant Pharmaceuticals (previously Bausch and Lomb). Dr. Ong played a key role in Bausch and Lomb's filing of new drug applications and its transformation into a global competitor in the specialty care sector. Previously, Dr. Ong worked at Pfizer Inc., developing drugs for diseases with high unmet medical need in various disease areas including respiratory, gastrointestinal, hepatology, and ophthalmology. Dr. Ong holds a medical degree from the University of London and an MBA from New York University Stern School of Business.

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 timing and outcome of PTC's regulatory strategy and process, including as it relates to PTC's submissions with the FDA, European Medicines Agency (EMA) and other regulatory bodies outside of the US or European Economic Area (EEA) and related regulatory reviews; PTC's ability to maintain its current marketing authorizations or obtain and maintain additional marketing authorizations; PTC's ability to work with the FDA to resolve the matters set forth in the Refuse to File letter PTC received in connection with its new drug application (NDA) for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD); the clinical utility and potential advantages of Translarna; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance of Translarna; PTC's estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; the

timing, results and conduct of PTC's clinical trials and studies, including statements regarding the timing of initiation, evaluation, enrollment and completion of the trials and studies and the period during which the results of the trials and studies will become available; 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 "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "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 forwardlooking statements it makes as a result of a variety of risks and uncertainties, including those related to: the timing and 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 NDA submission; whether the FDA, the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD or PTC's other clinical trials; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; whether patients and healthcare professionals may be able to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome, including whether Translarna may be accessed through a reimbursed importation pathway provided under German law and whether such pathway will minimize any access issues for German patients while maintaining a sustainable price; 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; 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; PTC's ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to obtain full marketing authorization in the EEA or obtain or maintain marketing authorizations in territories outside the EEA; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; 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 eligible patient base and commercial potential of Translarna and PTC's other product candidates; the outcome of ongoing or future clinical trials or studies; PTC's ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; 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 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 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:<u>http://www.prnewswire.com/news-releases/ptc-therapeutics-appoints-dr-tuyen-ong-as-chief-medical-officer-300257676.html</u>

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