January 9, 2017

European Commission Ratifies Positive CHMP Opinion for the Renewal of Translarna™ Marketing Authorization for the Treatment of Nonsense Mutation Duchenne Muscular Dystrophy

SOUTH PLAINFIELD, N.J., Jan. 9, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced that it received notification today from the European Commission (EC) of its adoption of a positive decision granting annual renewal of the conditional marketing authorization for Translarna™ (ataluren). The positive decision is based on the recent renewal recommendation by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). The authorization renewal allows the Company to market Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged five years and older in the 28 countries that are Member States of the European Union, as well as European Economic Area members Iceland, Liechtenstein and Norway. As a specific obligation of the renewal, the Company will conduct an additional trial of Translarna in nmDMD.

"As we continue towards our goal of providing Translarna to all who may benefit, we are pleased by the EC ratification of the recent CHMP positive opinion," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "This important regulatory milestone supports the continued growth of our sustainable ex-US business in both the European Union and countries that reference the authorization. Duchenne is a rapidly progressive disease and physicians need access to medicines, like Translarna, that have the potential to slow the devastating progression of this disorder."

About PTC Therapeutics, Inc.
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 process, including with respect to PTC's plan to conduct an additional trial of Translarna in nmDMD; our ability to continue commercial growth in the European Union and countries that may reference the Translarna marketing authorization in the European Economic Area; the clinical utility and potential advantages of Translarna; 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 "will," "plan," "expect," "target," "anticipate," "believe," "estimate," "intend," "may," "possible," "potential," "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 EEA, including whether the European Medicines Agency determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; the final design of the new nmDMD trial that PTC will undertake pursuant to the specific obligation associated with the marketing authorization and PTC's ability to enroll, fund and conduct such trial; the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including PTC's ability to resolve the matters set forth in the Refuse to File letter from the FDA or otherwise advance Translarna for the treatment of nmDMD in the United States (whether pursuant to the file over protest process or otherwise), 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; the outcome of ongoing or future clinical trials or studies in Translarna, in particular ACT CF; 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; 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 (or other delay or deceleration of the regulatory process), and PTC's ability to meet existing or future regulatory standards with respect to Translarna; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its 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/european-commission-ratifies-positive-chmp-opinion-for-the-renewal-of-translarna-marketing-authorization-for-the-treatment-of-nonsense-mutation-duchenne-muscular-dystrophy-300388180.html

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