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PTC Therapeutics Outlines Business Priorities for the Development and Commercialization of Translarna™ and Progress of the Company Pipeline

SOUTH PLAINFIELD, N.J., Jan. 11, 2016 /PRNewswire/ --Â PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a corporate update, which will be detailed at the company's presentation at the 34th Annual J.P. Morgan Healthcare Conference. Stuart W. Peltz, Ph.D., Chief Executive Officer will present the company's corporate strategy and anticipated 2016 milestones at 9:30 a.m. PST on Wednesday, Jan. 13, 2016. The presentation will be webcast live on the Events and Presentations page under the investor relations section of PTC Therapeutics' website at www.ptcbio.com.

Corporate Update

Commercial Highlights:

- Completed a landmark first year ex-U.S. launch of Translarna™ (ataluren) with 206 nonsense mutation Duchenne muscular dystrophy (nmDMD) patients on commercial therapy as of January 8th, including patients from both commercial sales and reimbursed early access programs.
- Translarna is now available on a commercial basis in 18 countries and PTC has a global commercial footprint of 46 countries either directly or through commercial partners.
- Preparations are underway for a potential 2016 launch of Translarna for nmDMD in the U.S.

Regulatory Highlights:

- Completed rolling NDA submission for Translarna to FDA and submitted Phase 3 ACT DMD results to EMA fulfilling the principal condition of the approval in the EU. Regulatory decisions are expected mid-2016.
- Following the submission of a variation for Translarna for nonsense mutation cystic fibrosis to the EMA in 2015, PTC is responding to questions and expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) in mid-2016.

Pipeline Highlights:

- Advancing PTC's 10 by 20 goal to expand Translarna's pipeline, investigational new drug applications (INDs) for aniridia and Dravet syndrome / CDKL5 have been filed.
- PTC and collaboration partners Roche and the SMA Foundation expanded the SMA program to include RG7916, an additional SMN2 splicing modifier. A Phase 1 study evaluating RG7916 was recently initiated in healthy volunteers. Results will inform the development path forward for the SMA program.
- Data expected in 2016 from Phase 1 oncology study in BMI1.

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, oncology and infectious diseases. 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 websiteÂ 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 of its regulatory process, including as it relates to PTC's submissions with the FDA, EMA and other regulatory bodies outside of the US or EEA and related regulatory reviews; 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 of Translarna for the treatment CF and other indications, as well its Phase 1 RG7916 and Phase 2 MOONFISH studies under its SMA collaboration with Roche and the SMA Foundation, and its Phase 1 study under its cancer stem cell program, 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.Â 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 forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD and other data with respect to the safety and efficacy of Translarna; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), 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 certain conditions and is also subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, including its ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries 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; expectations for regulatory approvals; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; 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.