January 9, 2017

PTC Therapeutics Provides Corporate Update and Outlines 2017 Strategic Priorities to Maximize the Global Value of Translarna™ and Advance its Innovative Pipeline

- Preliminary 2016 Translarna unaudited net sales of approximately $81M, a 140% increase vs. 2015 -
- Strategic update for Translarna regulatory path in the U.S. -
- Topline ACT CF data anticipated late first quarter 2017 -
- SMA program actively enrolling patients across SUNFISH and FIREFISH trials -
- RG7916 granted orphan-drug designation for the treatment of SMA -
- Translarna 2017 net sales guidance of $105M to $125M -

SOUTH PLAINFIELD, N.J., Jan. 9, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a corporate update, which will be detailed as part of the company presentation at the 35th Annual J.P. Morgan Healthcare Conference on Wednesday, January 11th at 7:30 am PT. Stuart W. Peltz, Ph.D., PTC's Chief Executive Officer, will present the company's 2017 strategic priorities, preliminary 2016 financial results and 2017 financial guidance. The presentation will be webcast live and available with the related slide deck on the Events and Presentations page under the investors section of PTC Therapeutics' website at www.ptcbio.com.

Commercial Highlights, Preliminary 2016 Unaudited Financial Results, and 2017 Guidance

- PTC expects to report Translarna (ataluren) net sales for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $81 million for 2016, an increase of 140% over the prior year and achieving the upper-end of guidance. This strong performance reflects rapid uptake, sustainable pricing, and high (> 90%) compliance to treatment.
- PTC expects to report year-end 2016 cash and cash equivalents of approximately $230 million.
- For 2017, PTC expects to achieve ex-U.S. Translarna nmDMD net sales of between $105 and $125 million, assuming current exchange rates, representing continued strong growth year-over-year of its sustainable DMD business. This is driven by both increased penetration into the over 25 countries where Translarna is currently available as well as continued geographic expansion into new territories.
- Non-GAAP operating expenses for 2017 are expected to be between $190 and $200 million excluding estimated non-cash stock-based compensation expense of approximately $35 million, for total operating expenses of approximately $225 to $235 million.
- PTC expects to finish 2017 with approximately $160 million of cash and cash equivalents.

Clinical and Regulatory Highlights

- Following multiple interactions with U.S. FDA officials and PTC's advisors, PTC plans to file the Translarna New Drug Application (NDA) for nmDMD over protest with the U.S. FDA in the first quarter of 2017. Feedback indicated this process, rather than continued appeal, is the best path forward for the current Translarna NDA to receive a full and fair review. Filing over protest is a procedural path permitted by U.S. FDA regulations that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission. PTC plans to supplement the current NDA with additional efficacy analyses utilized by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) in their recent renewal recommendation.
- The EMA's CHMP recommended the renewal of the conditional marketing authorization for Translarna (ataluren) for the treatment of nmDMD based on a continued positive benefit-risk assessment. As a specific obligation of the renewal, PTC will conduct an additional trial of Translarna in nmDMD.
- Top-line results of ACT CF are anticipated late in the first quarter of 2017. ACT CF is a Phase 3, international, multicenter, randomized, double-blind, placebo-controlled trial that is evaluating the absolute change in percent predicted forced expiratory volume in one second (FEV1) in patients with nonsense mutation cystic fibrosis (nmCF).
- The spinal muscular atrophy (SMA) program, a joint collaboration with Roche and the SMA Foundation, is expected to advance into two pivotal studies in 2017. SUNFISH and FIREFISH are both two part studies in childhood onset (Type 2/3) and infant onset (Type 1) SMA patients, respectively. Both studies are enrolling the initial dose escalation part of the study which will then transition to the pivotal part of the study evaluating efficacy. Commencement of the pivotal portion of either study will trigger a $20 million milestone payment to PTC from Roche. RG7916 was recently granted orphan-drug designation by the U.S. FDA.

Pipeline Highlights:

- Phase 2 proof-of-concept studies of Translarna in additional rare disease indications, including aniridia, MPS I, and Dravet/CDKL5, continue to progress. Proof-of-concept from these studies would further validate Translarna's potential as a precision medicine for a number of rare genetic disorders caused by a nonsense mutation.
- Clinical development of PTC596 is expected to progress into additional clinical studies in 2017. PTC596 is a novel, oral investigational drug that reduces the levels of BMI1, a protein required for cancer stem cell survival. An ongoing Phase 1 dose escalating study confirms that PTC596 is generally well tolerated at doses that achieved or exceed plasma concentrations in preclinical models.
- PTC's genetic disorders research organization is actively advancing lead optimization programs from its splicing platform focused on Huntington's disease and Familial Dysautonomia.

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; PTC's preliminary 2016 unaudited results, including (i) 2016 net sales of Translarna for the treatment of nmDMD and (ii) year-end 2016 cash and cash equivalents; PTC's financial guidance for 2017, including (i) net sales, (ii) non-GAAP and GAAP operating expenses, and (iii) ending cash and cash equivalents; the timing and outcome of PTC's regulatory process including, (i) 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 U.S., including its ability to supplement the NDA with additional efficacy analyses and submit its NDA for nmDMD with the FDA via the file over protest process in the first quarter of 2017 and (ii) the final determination by the European Commission with respect to renewal of the marketing authorization in the European Economic Area (EEA) for Translarna for the treatment of nmDMD and PTC's plan to conduct an additional Phase 3 randomized trial of Translarna in nmDMD; the clinical utility and potential advantages of Translarna; when top-line results of ACT CF will be available and reported; the timing, results and conduct of PTC's clinical studies of PTC596 and Translarna for the treatment of other indications, including statements regarding the timing of initiation, evaluation, enrollment and completion of the studies; any further advancement of either or both of the FIREFISH and SUNFISH studies under the joint SMA collaboration, including transition into the pivotal part of either study; the timing of a milestone payment, if any, to PTC from Roche; PTC's ability to continue to supply Translarna to patients, increase commercial penetration in countries where Translarna is currently available, and continue commercial expansion into new territories; 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 "preliminary," "guidance," "will," "plan," "expect," "target," "anticipate," "believe," "estimate," "intend," "may," "potential," "project," "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: the preliminary nature of PTC's 2016 financial results, which are subject to completion of its year-end audit; the assumptions underlying PTC's financial guidance for 2017; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Commission determines to approve the renewal of such authorization and whether the EMA 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 (if renewed) 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 NDA; the outcome of ongoing or future clinical trials or studies in Translarna, in particular ACT CF; events during, or as a result of, the SUNFISH or FIREFISH studies that could delay or prevent further advancement of the SMA program, including future actions or activities under the SMA joint development program; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; the EMA's determinations with respect to PTC's variation submission which seeks to add Translarna for the treatment of nmCF 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; 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 (or 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 scientific approach and general development progress; 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/ptc-therapeutics-provides-corporate-update-and-outlines-2017-strategic-priorities-to-maximize-the-global-value-of-translarna-and-advance-its-innovative-pipeline-300387565.html

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