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Phase 2 Data from Spinal Muscular Atrophy Program Presented at the 20th International Annual Congress of the World Muscle Society

- RG7800 demonstrated a favorable safety profile and was well tolerated over 12 weeks of treatment in patients with SMA -
- Up to two-fold increases in SMN protein versus baseline were observed in patients receiving treatment with RG7800 -

BRIGHTON, United Kingdom, Oct. 4, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that clinical data from the company's joint development program with Roche and the SMA Foundation in spinal muscular atrophy (SMA) were presented at the 20th International Congress of the World Muscle Society (WMS) in Brighton, U.K. Results from the first cohort of patients enrolled in the Phase 2 'MOONFISH' trial evaluating oral RG7800, a small molecule modifier of Survival Motor Neuron 2 (SMN2) splicing, were highlighted in a late breaking oral session. The presentation is titled "SMN2 splicing modifier RG7800 increases SMN protein in first study in SMA patients."

"We have now demonstrated, in two independent studies, that treatment with RG7800 shifts SMN2 splicing toward the production of full length SMN mRNA. Most importantly, in the MOONFISH trial we also observed relevant increases in SMN protein levels in whole blood in patients with SMA," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This is important because a two-fold increase of SMN protein levels has the potential to provide meaningful clinical benefit to SMA patients. SMA is a devastating disease with no marketed therapies currently available, and we remain focused on developing an oral therapy for the treatment of this disorder."

MOONFISH is a Phase 2 randomized, double-blind, placebo-controlled study investigating the safety, tolerability, pharmacokinetics and pharmacodynamics of RG7800 with a target enrollment of approximately 64 adult and pediatric patients with SMA. Results from the first cohort that included 13 adult and adolescent SMA patients demonstrated that SMN protein can be increased with RG7800, providing proof of mechanism for oral small molecule SMN2 splicing modifiers. Up to three-fold increases in the ratio of full length SMN2 mRNA to SMN2Δ7 mRNA and up to two-fold increases in SMN protein were observed versus baseline, as measured in whole blood. RG7800 was well tolerated over 12 weeks at a dose of 10 mg once daily.

RG7800 is an orally available small molecule being investigated for its ability to selectively modify the alternative splicing of the SMN2 gene, which is present both in healthy individuals and SMA patients, towards the production of full length mRNA. Preclinical studies in animal models of SMA demonstrated an increase in functional full length SMN protein levels with significant efficacy benefits on survival and motor function. In a Phase 1 clinical study in healthy volunteers, a dose-dependent effect on SMN2 alternative splicing was observed. Dosing in the Phase 2 MOONFISH trial was suspended in April 2015 as a precautionary measure, while a non-clinical safety finding observed in a longer-term animal study is investigated.

The SMA program was initially developed by PTC Therapeutics in partnership with the SMA Foundation. The SMA Foundation was established in 2003 to accelerate the development of a treatment for SMA. In November 2011, Roche gained an exclusive worldwide license to the PTC / SMA Foundation SMN2 alternative splicing program. The development of RG7800 is being executed by Roche and overseen by a joint steering committee with members from PTC, Roche, and the SMA Foundation.

About Spinal Muscular Atrophy (SMA)
Spinal Muscular Atrophy (SMA) is a genetic neuromuscular disorder that is the leading genetic cause of mortality in infants and toddlers caused by a missing or defective survival of motor neuron 1 (SMN1) gene, which results in reduced levels of SMN protein. The homologous SMN2 gene is predominantly spliced to a shortened mRNA, and only produces small amounts of functional SMN protein. Insufficient levels of SMN protein are responsible for the loss of motor neurons within the spinal cord leading to muscle atrophy and death in infants and toddlers in its most severe form. It is estimated that this devastating disease affects 1 in every 11,000 children born. There are no marketed therapies for SMA.

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, 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 website www.ptcbio.com

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Forward Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; our expectations with respect to the development and regulatory status of our product candidate and joint development program with Roche and the SMA Foundation directed against SMA; the timing and conduct of clinical trials and studies under PTC's SMA collaboration with Roche and the SMA Foundation, including the Phase 2 MOONFISH study; our strategy, future operations, future financial position, future revenues or projected costs; and 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 forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to the initiation, conduct and availability of data from clinical trials and studies; expectations for regulatory approvals; PTC's scientific approach and general development progress; 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. 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 release except as required by law.