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Spinal Muscular Atrophy Program Advances with Additional Product Candidate Entering Clinical Development

-Phase 1 Study Initiated in Healthy Volunteers with New SMN2 Splicing Modifier - RG7916-

SOUTH PLAINFIELD, N.J., Jan. 7, 2016 /PRNewswire/ --Â PTC Therapeutics, Inc. (Nasdaq: PTCT) today announced that RG7916, an additional SMN2 splicing modifier from the company's joint development program with Roche and the SMA Foundation in spinal muscular atrophy (SMA), has entered clinical development.Â RG7916 is designed to shift SMN2 pre-mRNA splicing toward the production of full length SMN mRNA. Â A Phase 1 study in healthy volunteers has been initiated to investigate the safety, tolerability, pharmacokinetics and pharmacodynamics of RG7916.

"We are pleased to initiate clinical development of a second candidate in our SMA collaboration," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The goal of this Phase 1 study is to understand more about the safety and activity of RG7916 and be in a position to compare the profiles of each of our development compounds to determine the best path forward for our SMA program.Â SMA is the most common genetic cause of infant mortality and one of the most common rare diseases. Â Currently there are no available therapies to treat the underlying cause of the disease. PTC and our collaboration partners are committed to advancing potential solutions for SMA patients through our SMN2 alternative splicing program."

Two compounds are currently in clinical development within the SMA program, RG7800 and RG7916. The most advanced compound, RG7800, is the subject of a Phase 2 randomized, double-blind, placebo-controlled trial called Moonfish in adult and pediatric patients with SMA.Â Dosing in the Moonfish trial was suspended in April 2015 and the trial was placed on clinical hold to investigate a non-clinical safety finding observed in a longer-term animal study.

The SMA program was initially developed by PTC Therapeutics in partnership with the SMA Foundation in 2006 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 and RG7916 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 truncated 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 its most severe form. It is estimated that this devastating disease affects 1 in every 11,000 children born. Currently, there are no therapies available for SMA.Â

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:Â Â Â Â
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 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 1 study of RG7916 and 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; our SMA collaboration; our 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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/spinal-muscular-atrophy-program-advances-with-additional-product-candidate-entering-clinical-development-300200907.html

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