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RG7916 Granted Orphan Drug Designation in the U.S. for the Treatment of Spinal Muscular Atrophy

SOUTH PLAINFIELD, N.J., Jan. 6, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to RG7916 for the treatment of patients with Spinal Muscular Atrophy (SMA). RG7916 is part of PTC's joint development program in SMA with Roche and the SMA Foundation (SMAF). SMA is a rare genetic disorder that results in neuromuscular disability beginning in infancy and is the leading inherited cause of mortality in infants and young children.

RG7916 is an oral small molecule splicing modifier that directly targets the underlying molecular deficiency of SMA by modulating *SMN2* splicing to increase expression of stable full-length SMN protein from the *SMN2* gene. RG7916 is currently under investigation in two clinical studies: SUNFISH, a trial in childhood onset (Type II/III) SMA patients, and FIREFISH, a trial in infant onset (Type I) patients.

"We are dedicated to the development of innovative treatments for patients, particularly in genetic disorders such as SMA," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "This orphan drug designation further supports the need for additional treatments for this devastating disease. As an oral small molecule with broad exposure, RG7916 has the potential to impact every aspect of this disease and provide hope to many patients."

In the U.S., orphan drug designation is granted by the FDA's Office of Orphan Products Development to promote the development of products that may offer therapeutic benefits for diseases with a prevalence of fewer than 200,000 individuals per year. Orphan drug designation provides opportunities for grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States, if granted FDA approval.

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 these compounds is being executed by Roche and overseen by a joint steering committee with members from PTC, Roche, and the SMA Foundation.

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: any advancement of the joint development program in SMA with PTC, Roche, and SMAF, in particular

as related to the timing, enrollment, completion and evaluation of the Phase 2 clinical studies of RG7916 in SMA patients and the period during which the results of the studies will become available; any benefits that may be realized by reason of the orphan drug designation; the clinical utility and potential advantages of RG7916, including its potential to impact every aspect of the disease; the timing and outcome of PTC's regulatory strategy and process; PTC's strategy, future expectations, plans and prospects, 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 "potential," "will," "promise," "expect," "plan," "target," "anticipate," "believe," "estimate," "intend," "may," "project," "possible," "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, enrollment, conduct and availability of data from either the SUNFISH or FIREFISH studies and the outcome of such studies; events during, or as a result of, these studies that could delay or prevent further development of RG7916, including future actions or activities under the SMA joint development program; 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.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products, including with respect to PTC's joint development program in SMA with Roche and the SMAF. There are no guarantees that any product candidate under the joint development program will receive regulatory approval in any territory or prove to be commercially successful.

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/rg7916-granted-orphan-drug-designation-in-the-us-for-the-treatment-of-spinal-muscular-atrophy-300386964.html

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