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RG7916 Increased SMN Protein Production in SUNFISH Clinical Trial in Patients with Type 2/3 Spinal Muscular Atrophy

- Data presented at the 22nd International World Muscle Society (WMS) Congress -

SOUTH PLAINFIELD, N.J., Oct. 3, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced data from Part 1 of the SUNFISH trial of RG7916 in Type 2/3 spinal muscular atrophy (SMA) patients. RG7916 is part of PTC's joint development program in SMA with Roche and the SMA Foundation (SMAF). The data were presented at the 22nd International World Muscle (WMS) Congress in St. Malo, France. An interim analysis of the five cohorts treated with RG7916 for 28 days or longer demonstrated an exposure-dependent increase in SMN protein. SMA is characterized by reduced levels of SMN protein, motor neuron loss, and muscle atrophy. It is the leading genetic cause of mortality in infants and young children. To date, RG7916 remains well-tolerated in patients at all doses and there have been no drug-related safety findings leading to withdrawal.

"We are excited to report that in SMA patients who are deficient in SMN protein, RG7916 treatment led up to a median 2.5-fold increase in SMN protein," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "SMN protein is a critical factor that is needed both in the central nervous system and in a number of other tissues and organs throughout the body. Increasing SMN protein levels throughout the body has the potential to impact every aspect of this disorder. These results have allowed us to determine the dose for the pivotal phase of the SUNFISH trial to evaluate the efficacy and safety of RG7916 versus placebo."

SUNFISH is a double-blind, two-part, placebo-controlled trial. Part 1 of SUNFISH enrolled patients with Type 2/3 SMA to evaluate safety, tolerability, and PK/PD of several RG7916 dose levels. The pivotal SUNFISH Part 2, in non-ambulant patients with Type 2/3 SMA, will evaluate safety and efficacy of the RG7916 dose level selected from Part 1.

The U.S. Food and Drug Administration granted orphan drug designation and fast track designation to RG7916 for the treatment of patients with SMA earlier this year. RG7916, an orally administered small molecule, directly targets the underlying cause of SMA by modulating SMN2 splicing to increase expression of full-length SMN2 mRNA and increases SMN protein levels.

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 novel medicines using our expertise in RNA biology. 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. Since its founding nearly 20 years ago, PTC's mission has focused on developing treatments to fundamentally change the lives of patients living with rare genetic disorders. The company was founded in 1998 and is headquartered in South Plainfield, New Jersey. 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 of 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; whether and when a milestone payment to PTC from Roche may be triggered; 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.

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