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Preliminary Phase 2 Data from Spinal Muscular Atrophy Program Presented at CureSMA Conference

-- RG7916 demonstrated ~400% increase in SMN2 full length/Δ7 mRNA ratio --
-- RG7916 demonstrated a favorable preliminary safety profile and was generally well tolerated in Type 2/3 SMA patients --

SOUTH PLAINFIELD, N.J., July 1, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the presentation of preliminary clinical data from the company's joint development program with Roche and the SMA Foundation in spinal muscular atrophy (SMA) at the Cure SMA Meeting in Orlando, FL. Preliminary results from an early analysis of Part 1 of the Phase 2 SUNFISH trial evaluating oral RG7916, a small molecule modifier of Survival Mo tor Neuron 2 (SMN2) splicing, were highlighted in an oral presentation titled "Clinical Studies of RG7916 in Patients with Spinal Muscular Atrophy: Study Update." SMA is a rare genetic disorder that results in neuromuscular disability beginning in infancy and is the leading genetic cause of mortality in infants and young children.

"The increase in SMN2 full length transcript in SMA patients is promising and confirms that RG7916 targets the underlying cause of the disease," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The low levels of SMN protein in SMA patients affect multiple tissues throughout the body, including muscles, bones and nerves. We believe that the ease of administration of an oral therapy and its broad tissue distribution give RG7916 the potential to address the complete spectrum of functional deficits observed in SMA patients. We look forward to advancing RG7916 into pivotal clinical trials in the second half of the year."

Preliminary results from an early analysis from the ongoing Part 1 of the RG7916 SUNFISH study in Type 2/3 SMA patients demonstrated a dose-dependent increase in SMN2 full length/Δ7 mRNA ratio of ~ 400% versus baseline, as measured in whole blood. These results provided proof of mechanism for oral small molecule SMN2 splicing modifiers. No drug-related adverse events leading to withdrawal have been observed to date for RG7916.

SUNFISH is a two-part phase 2 clinical study. Part 1 is a double-blind, placebo-controlled, randomized, exploratory dose-finding study in Type 2/3 pediatric and adult SMA patients. The primary objective of the first part of the study is to evaluate the safety, pharmacokinetics, and pharmacodynamics of RG7916 in patients, and to select the dose for the second part of the study. The pivotal second part is a double-blind, placebo-controlled, randomized, confirmatory study in Type 2/3 SMA patients followed by an open-label extension. The primary objective of the pivotal second part of the study is to evaluate the safety and efficacy of RG7916 compared to placebo. SUNFISH is one of three ongoing clinical trials of RG7916, along with FIREFISH and JEWELFISH.

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

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' 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.