Updated Preliminary Data from SMA FIREFISH Program in Type 1 Babies Presented at the CureSMA Conference

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- risdiplam (RG7916) is well tolerated at all dose levels with no drug-related safety findings -

SOUTH PLAINFIELD, N.J., June 16, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the presentation of updated interim clinical data from Part 1 of the FIREFISH study investigating risdiplam (RG7916) in babies with Type 1 Spinal Muscular Atrophy (SMA), at the 22nd Annual SMA Researcher Meeting. The data presented by Dr. Baranello demonstrated that at Day 182, over 90% of the babies achieved a greater than 4-point increase in CHOP-INTEND score compared to baseline. The CHOP-INTEND data were further supported by video footage presented by Dr Baranello demonstrating antigravity movements, the ability to control their head, roll, or sit in babies participating in FIREFISH. Part 2 of the pivotal FIREFISH study is ongoing. The SMA program is a collaboration between PTC, Roche, and the SMA Foundation.

"We are delighted that up to 6.5-fold increase of protein production has translated into clinical impact for these babies in the FIREFISH study," said Stuart W. Peltz, Ph.D. Chief Executive Officer of PTC Therapeutics. "The survival data and CHOP-INTEND scores are very promising, since babies with Type 1 SMA typically do not experience functional motor milestone improvement based on natural history. We look forward to sharing updates for the programs as the data further develop at upcoming medical meetings."

The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) is a test designed to measure motor milestone development of patients with SMA Type 1. In the FIREFISH study the median increases in CHOP-INTEND scores were 5.5 points (n=20) at Day 56, 12.5 points (n=16) at Day 119, and 14 points (n=11) at Day 182 of treatment. The proportion of patients achieving greater than a four-point increase from baseline in CHOP-INTEND scores was 75% at Day 56 (n=20), 94% at Day 119 (n=16), and 91% (n=11) at Day 182. In addition, risdiplam has been well tolerated at all dose levels and to date there have been no drug-related safety findings leading to withdrawal. Also, no babies have required a tracheostomy or permanent ventilation since study initiation and no baby has lost the ability to swallow. Median age of patients at first dose in the FIREFISH trial is 6.7 months; oldest patient in the trial is currently 23.8 months old.

"I am impressed by the clinical data and the changes reported by the patients' families," stated Dr. Giovanni Baranello, Fondazione Istituto Neurologico Carlo Besta in Milan, Italy. "Data on motor function seem more encouraging when we consider that we are seeing motor function improvements and milestones achievement at this early stage of the study, which was essentially a dose-finding study and most of the infants included have received their first dose after the age of 5 months. It is exciting to see evidence of clinical benefit from a systemic oral treatment for SMA."

Risdiplam is an investigational splicing modifier targeting the survival motor neuron 2 (SMN2) RNA, restoring a functional transcript. Risdiplam is taken orally, crosses the blood brain barrier, and shows systemic distribution to the organs that are affected by low levels of SMN protein.

Other presentations included an analysis of data from Part 1 of the SUNFISH study which demonstrated that risdiplam administration resulted in a dose-dependent increase in SMN protein levels up to 3.5-fold; pharmacodynamic data from the JEWELFISH trial; and preclinical data demonstrating SMN protein production and distribution, as well as the development of the SMA Independence Scale (SMAIS).

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.

About the SMA Clinical Trials
FIREFISH: An open-label, two-part clinical trial. Part 1 was a dose escalation study in 21 infants. The primary objective of Part 1 was to assess the safety profile of risdiplam in infants and determine the dose for Part 2. Part 2 is a single-arm study with the dose selected in Part 1 in approximately 40 infants with Type 1 SMA for 24 months, followed by an open-label extension. This study is recruiting globally.

SUNFISH: A double-blind, two-part, placebo-controlled trial. Part 1 enrolled patients with Type 2 or 3 SMA to evaluate safety, tolerability, and PK/PD of several risdiplam dose levels. The pivotal SUNFISH Part 2, in non-ambulant patients with Type 2 or 3 SMA, is evaluating safety and efficacy of the risdiplam dose level selected from Part 1 for 24 months, followed by an open label extension. This study is recruiting globally.

JEWELFISH: An ongoing, exploratory, open-label study to establish the safety and tolerability of risdiplam in people who have previously participated in a study with another therapy targeting SMN2 splicing.

About the SMA collaboration
The SMA program was initially developed by PTC Therapeutics in partnership with the SMA Foundation in 2006. In November 2011, Roche gained an exclusive worldwide license to the PTC/SMA Foundation SMN2 alternative splicing program. The development of risdiplam RG7916 is being executed globally by Roche, including in the US through Genentech, a member of the Roche group. The SMA program is overseen by a Joint Steering Committee with members from PTC, Roche, and the SMA Foundation.

About PTC Therapeutics
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder

products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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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; 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" sections of PTC's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K 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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