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Preliminary Data from FIREFISH trial in Type 1 SMA Infants Presented at the International Scientific Congress on Spinal Muscular Atrophy

SOUTH PLAINFIELD, N.J., Jan. 27, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the presentation of early interim data from Part 1, the dose-finding portion of the FIREFISH study. FIREFISH is a two-part seamless, open-label, multi-center study to investigate the safety and efficacy of RG7916 in infants and babies with Type 1 SMA. RG7916 has been safe and well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. In addition, data on the ability to swallow and requirements for tracheostomy or permanent ventilation, together with overall survival were also presented. Previously published natural history data indicate that in a comparable historic cohort the median age of event-free survival for SMA Type 1 infants to be between 8 and 10.5 months[1,2]. The presentation was given by Dr. Giovanni Baranello, Fondazione Istituto Neurologico Carlo Besta in Milan, Italy, at the International Scientific Congress on spinal muscular atrophy in Kraków, Poland, and will be made available via a link under the investor relations section of the PTC website. (www.ptcbio.com/smaeurope)

"It is exciting to show for the first time that an oral small molecule demonstrates early signs of clinical benefit," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We believe a drug that distributes to both the CNS and peripheral tissues can provide an important benefit to children suffering from this devastating, fatal disease. We anticipate that the trial will transition to the pivotal portion in the coming months."

FIREFISH (NCT02913482) is a multi-center, open-label, seamless pivotal study evaluating the safety and efficacy of RG7916 in babies aged 1-7 months at enrollment with Type 1 SMA and two SMN2 gene copies. The exploratory Part 1 (n=8-24) is assessing the safety, tolerability, pharmacokinetics and pharmacodynamics of RG7916 at different dose levels. In Part 1, patients receive RG7916 for at least 4 weeks (or 2 weeks after steady-state is achieved) of daily administration; patients then enter an extension phase with RG7916. The confirmatory Part 2 (n=40) will assess the safety and efficacy of RG7916 at the dose level selected from Part 1 over 24 months. The primary endpoint for Part 2 is the proportion of infants sitting without support for 5 seconds, assessed by the Gross Motor Scale of the BSID-III, after 12 months of treatment.

"This early interim analysis of survival and the delay to milestone events that are hallmarks of the progression of SMA in infants are promising," stated Dr. Giovanni Baranello, Fondazione Istituto Neurologico Carlo Besta in Milan, Italy. "These data, in conjunction with earlier poster presentations at the Congress from the trials in patients with type 2/3 SMA, are supportive of safety and sustained increase in SMN protein levels. Clinical trials in SMA patients require a globalization of clinical research; particularly, it is essential for a coordinated multidisciplinary team to support the babies and their families and to ensure the best standard of care to promote the higher benefit to affected children from potential new treatments."

In addition to the oral presentation, three posters were on display during the Congress: updated pharmacodynamic and safety data from SUNFISH Part 1, preliminary evidence for pharmacodynamic effects of RG7916 in JEWELFISH, and preclinical data demonstrating the relationship between central and peripheral SMN protein increase upon treatment with RG7916. The data presented demonstrate systemic and dose-dependent increase of SMN protein levels. The data from mice and other species suggest that SMN protein level increases seen in the blood of patients following RG7916 treatment reflect SMN protein level increases in the CNS, muscle and other key issues affected in SMA. In addition, RG7916 has been safe and well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal.

The U.S. Food and Drug Administration granted orphan drug designation to RG7916 for the treatment of patients with SMA. RG7916 directly targets the underlying molecular deficiency of SMA by modulating SMN2 splicing to increase expression of full-length SMN2 mRNA from the SMN2 gene. An interim analysis from the first part of SUNFISH 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. To date, RG7916 remains well-tolerated in patients at all doses and there have been no drug-related safety findings leading to withdrawal.

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.

1 Kolb S, et al. Amer Neuro Assoc 2017; 883-891
2 Finkel R, et al. Amer Acad Neur 2014; 810-817

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 is a dose escalation study in at least 8 infants for a minimum of 4 weeks. The primary objective of Part 1 is to assess the safety profile of RG7916 in infants and determine the dose for Part 2. Part 2 is a single-arm study in approximately 40 infants with Type 1 SMA for 24 months, followed by an open-label extension.

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 RG7916 dose levels. The pivotal SUNFISH Part 2, in non-ambulant patients with Type 2 or 3 SMA, will evaluate safety and efficacy of the RG7916 dose level selected from Part 1.

JEWELFISH: An exploratory, open-label study to establish the safety and tolerability of RG7916 in people who have previously participated in a study with another therapy targeting SMN2 splicing.

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 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; 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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