FIREFISH Transitions into Pivotal Phase

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- First patient dosed in Type 1 Spinal Muscular Atrophy Program -

SOUTH PLAINFIELD, N.J., March 15, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the FIREFISH study of RG7916 in Type 1 spinal muscular atrophy (SMA) patients has transitioned into its pivotal phase with the dosing the first patient. Spinal muscular atrophy is a rare neuromuscular disorder that is caused by reduced levels of the SMN protein which leads to the loss of motor neurons and results in progressive muscle weakness. Type 1 is the most severe form of SMA and generally results in death before age two. RG7916 is an investigational splicing modifier targeting the survival motor neuron 2 (SMN2) RNA, restoring a functional transcript. RG7916 is taken orally, crosses the blood brain barrier, and shows systemic distribution to the organs that are affected by low levels of SMN protein. The SMA program is a collaboration between PTC, Roche, and the SMA Foundation.

"We are pleased that the first infant has been dosed in this pivotal trial," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "It is especially encouraging that preliminary data from the first part of the FIREFISH study demonstrated that RG7916 was well tolerated at all doses, and that there have been no drug-related safety findings leading to withdrawal. A treatment that is delivered orally with systemic distribution has the potential to benefit the central nervous system as well as other parts of the body that are affected by low SMN protein."

FIREFISH is a global, open-label, single-arm study. In the pivotal part of the study, the efficacy and safety of RG7916 at the dose level selected from Part 1 will be evaluated over 24 months. Part 2 will be conducted in approximately 40 infants with Type 1 SMA, followed by a long-term extension. The primary objective of the study is to evaluate the proportion of infants sitting without support for 5 seconds, assessed by the Bayley Gross Motor Scale, after 12 months of treatment.

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. Recently presented preliminary data from Part 1 of the study demonstrated that RG7916 was well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. Additionally, no patient lost the ability to swallow and no patient has required tracheostomy or reached permanent ventilation.

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. Roche leads the global development of RG7916, 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 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, Inc.
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 disease 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. For more information, 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 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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