Preliminary Clinical Data from FIREFISH Trial in Type 1 SMA Patients Presented at the American Academy of Neurology Annual Meeting

April 25, 2018

SOUTH PLAINFIELD, N.J., April. 24, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the presentation of preliminary data from Part 1, the dose finding portion, of the ongoing FIREFISH clinical trial. The open-label trial is evaluating RG7916, an oral survival motor neuron 2 (SMN2) splicing modifier, in Type 1 SMA patients. Data from Part 1 were presented at the 2018 American Academy of Neurology Annual Meeting and demonstrated that RG7916 has been well tolerated at all dose levels and to date there have been no drug-related safety findings leading to withdrawal. In addition, no babies have required a tracheostomy or permanent ventilation since study initiation and no baby has lost the ability to swallow. The median age of first dose was 6.7 months and babies have received RG7916 for a duration of up to 14.8 months. Recruitment is ongoing globally for the pivotal second part of the FIREFISH study.

"RG7916 continues to demonstrate compelling results in SMA patients and we look forward to making further gains to combat this devastating, fatal disease," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We are encouraged by this promising data as we continue our ongoing global recruitment efforts for the SMA programs."

FIREFISH is an ongoing, global multi-center, open-label, seamless Phase 2 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. Data from 21 patients from the completed Part 1 portion of this study suggested that RG7916 produced a dose-dependent increase in SMN protein levels in babies with Type 1 SMA.

"SMA Type 1 is a devastating disease in which babies rarely live past the second year of life without permanent nutritional and/or ventilatory support," stated Dr. Giovanni Baranello, Fondazione Istituto Neurologico Carlo Besta in Milan, Italy. "This early data on the infants' ability to swallow and lack of need of permanent ventilatory support is very encouraging."

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 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 for a minimum of 4 weeks. The primary objective of Part 1 was to assess the safety profile of RG7916 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 RG7916 dose levels. The pivotal SUNFISH Part 2, in non-ambulant patients with Type 2 or 3 SMA, is evaluating safety and efficacy of the RG7916 dose level selected from Part 1. This study is recruiting globally.

JEWELFISH: An ongoing, 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 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.

For More Information:

Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167

jbaj@ptcbio.com

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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