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Spinal Muscular Atrophy Program Advances into Phase 2 Clinical Studies in SMA Patients with RG7916

- Initiated SUNFISH study to evaluate RG7916 in Type 2/3 SMA patients -
- FIREFISH study in Type I SMA patients expected to begin in the coming months -

SOUTH PLAINFIELD, N.J., Oct. 20, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that its joint development program in Spinal Muscular Atrophy (SMA) with Roche (SIX: RO, ROG; OTCQX: RHHBY) and the SMA Foundation (SMAF) initiated a Phase 2 study in pediatric and adult Type 2/3 SMA patients. The study, named SUNFISH, is a two-part study investigating the safety, tolerability and efficacy of RG7916, an oral small molecule survival motor neuron 2 (SMN2) splicing modifier. The first part of the study will evaluate safety and tolerability through escalating doses of RG7916. After dose selection, the study will transition into the pivotal second part evaluating the efficacy of RG7916. Initiation of the pivotal second part of the study is expected to begin in 2017 and will trigger a $20 million milestone payment to PTC from Roche. A similarly designed two-part study to evaluate RG7916 in Type I SMA patients is expected to begin in the coming months. 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.

"We are excited to initiate clinical studies in SMA patients with RG7916 and advance our Spinal Muscular Atrophy program forward," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "An oral small molecule splicing modifier has the potential benefit of systemic exposure to promote SMN protein in all affected organs and tissues. This attribute, along with an ease of administration, provides promise for this program to have a meaningful benefit for SMA patients."

SUNFISH is a two-part clinical study. Part one is a double-blinded, placebo-controlled, randomized, exploratory dose-finding study in approximately 36 Type 2 and Type 3 pediatric and adult SMA patients for a minimum of 12 weeks. 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-blinded, placebo-controlled, randomized, confirmatory study in approximately 150 Type 2 and Type 3 SMA patients for up to 24 months, followed by an open-label extension. The primary objective of the pivotal second part of the study is to evaluate the efficacy of RG7916 compared to placebo.

A trial in Type I SMA patients, named FIREFISH, is planned to initiate in the coming months. FIREFISH is also planned to be a two-part study. The first part is an open-label, dose-escalation study in at least eight infants for a minimum of four weeks. The primary objective of part one of the study is to assess the safety profile of RG7916 in infants and determine the dose for part two. The second part of FIREFISH is expected to be an open-label, single-arm study in approximately 40 infants with Type I SMA for 24 months, followed by an open-label extension. The primary objective of the second part of the study will be to assess the efficacy of RG7916 at the selected dose after 12 months of treatment.

RG7916 directly targets the underlying molecular deficiency of SMA by modulating *SMN2* splicing to increase expression of stable full-length SMN2 mRNA from the *SMN2* gene. A healthy volunteer study was recently completed and the preliminary results demonstrate that RG7916 increased the production of full-length SMN2 mRNA further demonstrating proof of mechanism for oral small molecule SMN2 splicing modifiers. RG7916 was also well tolerated.

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, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. 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. PTC plans to continue to develop these compounds both on its own and

through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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 initiation, evaluation, enrollment and completion 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 whether oral small molecule splicing modifier may promote SMN protein in all affected organs and tissues and whether this attribute, along with an ease of administration, may have a meaningful benefit for SMA patients; 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.