Risdiplam Spinal Muscular Atrophy Data Demonstrating Continued Benefit Presented at World Muscle Society Congress

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- Risdiplam-treated type 1 babies with SMA continue to achieve major motor milestones-

- Risdiplam-treated patients in SUNFISH demonstrate clinically meaningful increases in muscle function/performance compared to natural history -

-All clinical trials involving risdiplam continue to demonstrate strong safety profile -

-Filing for broad labels in 2019 (US) and 1H'20 (EU) -

SOUTH PLAINFIELD, N.J., Oct. 2, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced data demonstrating the ongoing benefit of risdiplam (RG7916) for the treatment of all types of spinal muscular atrophy (SMA) at the 24th International Annual Congress of the World Muscle Society. Presentations include data from the FIREFISH, SUNFISH, and JEWELFISH clinical trials. The SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

"We are excited that risdiplam has the potential to enter the market with a best-in-class profile for patients with all types of SMA," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "These data continue to demonstrate the disease-modifying properties of risdiplam across a broad range of ages and infants receiving the drug are continuing to experience improved motor outcomes. Importantly, these results help validate PTC's splicing platform that is currently being used in other programs."

Data from Part 1 of the FIREFISH clinical trial demonstrated that after 16 months of treatment, 82% (14/17) of high-dose patients had a CHOP-INTEND score ≥40. 86% (18/21) of all infants were event-free after receiving risdiplam for 16 months. No infant has required tracheostomy or reached permanent ventilation. The primary objective of FIREFISH Part 1 was to assess the safety profile of risdiplam in infants and determine the dose for Part 2.

In patients in Part 1 of the SUNFISH clinical trial, risdiplam treatment led to a median two-fold increase in blood SMN protein levels after four weeks of treatment, sustained for at least 12 months. In the natural history cohort, patients did not experience any change in SMN protein levels over the same period. Patients receiving risdiplam in SUNFISH Part 1 showed a clinically meaningful increase in total MFM32 score including a broad range of ages and functional status at baseline compared with natural history, independent of age and disease severity. SUNFISH Part 1 is focused on the safety, tolerability, pharmacokinetics, pharmacodynamics and exploratory efficacy in patients with type 2 or 3 SMA between 2 and 25 years old.

Data from 45 patients in the JEWELFISH trial demonstrated a sustained, greater than two-fold increase in median SMN protein versus baseline over 12 months of treatment. Patients in the JEWELFISH study have previously been treated with nusinersen or other therapies.

In August, the first patient was enrolled in the RAINBOWFISH trial, an open-label, single-arm, international, multi-center clinical study to investigate the efficacy, safety, pharmacokinetics and pharmacodynamics of risdiplam in infants with genetically diagnosed SMA who are not yet presenting symptoms.

Risdiplam has been well tolerated at all dose levels and in all clinical studies. To date, there have been no drug-related safety findings leading to withdrawal from any study.

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 risdiplam
Risdiplam is an investigational medicine being studied in a broad range of patients with SMA from 1 month to 60 years of age. It is designed to provide sustained increase in SMN protein centrally and peripherally through daily dosing and is being evaluated for its potential ability to help the SMN2 gene produce more functional SMN protein throughout the body.

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 PK, PD, safety and tolerability 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 now fully enrolled.

SUNFISH: A double-blind, two-part, placebo-controlled trial. Part 1 enrolled patients with type 2 or 3 SMA to evaluate the 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 now fully enrolled.

JEWELFISH: is an ongoing, multicenter, open-label study to assess the safety, tolerability and PK/PD relationship of once-daily oral administration of risdiplam in patients aged from 6 months to 60 years with SMA previously enrolled in Study BP29420 (MOONFISH) with the splicing modifier RO6885247 or received previous treatment with nusinersen (SPINRAZA®), olesoxime or onasemnogene abeparvovec (ZOLGENSMA®).

RAINBOWFISH: An open-label, single-arm, multicenter study to investigate efficacy, safety, pharmacokinetics and pharmacodynamics of risdiplam in newborns with genetically diagnosed pre-symptomatic SMA, aged from birth to 6 weeks old (at first dose).

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, 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. PTC's ability to globally commercialize products 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. To learn more about PTC, please visit us on www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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Forward-Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, 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 clinical studies of risdiplam in SMA patients and the period during which the results of the studies will become available; the clinical utility and potential advantages of risdiplam, including its potential to impact every aspect of the disease; the timing and outcome of the regulatory strategy and process for risdiplam, including any potential regulatory submissions; 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 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 risdiplam, 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 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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