Risdiplam (RG7916) Pivotal SUNFISH Study Demonstrates Statistically Significant Improvement for Patients with Type 2/3 Spinal Muscular Atrophy

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- Study meets primary endpoint for patients living with type 2/3 SMA -

- Risdiplam has been well tolerated and there have been no drug-related safety findings leading to withdrawal from the study -

SOUTH PLAINFIELD, N.J., Nov. 11, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced positive results from part 2 of SUNFISH demonstrating that the study met its primary endpoint of change from baseline after 1 year of treatment with risdiplam compared to placebo as measured by the Motor Function Measure 32, a scale monitoring severity and progression of fine and gross motor function in patients with a neuromuscular disease such as spinal muscular atrophy (SMA). Risdiplam has been well tolerated and no treatment-related safety findings leading to withdrawal have been seen in any risdiplam trial to date. Data from part 2 of the SUNFISH study will be presented at an upcoming medical congress.

"We are very pleased with the results of the SUNFISH study and are excited to move one step closer to bringing risdiplam globally to all patients living with SMA. The SUNFISH trial shows that risdiplam continues to demonstrate its disease-modifying properties and compelling safety profile," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "The results from the study will be shared first with regulators globally and then will be presented at a SMA conference early next year. We believe that risdiplam has the potential to enter the market with a best-in-class profile for patients with all SMA types. We are particularly grateful to the SMA community, the patients and investigators who participated in the trials."

Risdiplam (RG7916), is an investigational, oral, first-in-class, mRNA splicing modifier for the treatment of SMA. SUNFISH is 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, evaluated safety and efficacy of the risdiplam dose level selected from part 1 for 24 months, followed by an open label extension. Patients from 2-25 years of age were enrolled in the study, representing the broad real-world spectrum of patients living with SMA. The SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

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 birth 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. Risdiplam is also being studied in a clinical trial for patients with type 1 SMA, called FIREFISH, in pre-symptomatic babies, RAINBOWFISH and in patients who have been in previous clinical trials for SMA, JEWELFISH.

About PTC Therapeutics, Inc.
PTC is a science-driven, 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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