Risdiplam (RG7916) Pivotal FIREFISH Study Demonstrated Statistically Significant Improvement in Infants with Type 1 Spinal Muscular Atrophy

January 23, 2020

- Study meets primary endpoint in patients living with type 1 SMA -
- To date, more than 400 patients have been treated with risdiplam across all studies, with no treatment related safety findings leading to study withdrawal in any risdiplam trial -
- Risdiplam PDUFA expected May 24, 2020 -

SOUTH PLAINFIELD, N.J., Jan. 23, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced positive topline results from part 2 of FIREFISH demonstrating that the study met its primary endpoint of proportion of infants who are sitting without support after 12 months of treatment. The pivotal study assessed the efficacy of risdiplam (RG7916) in infants with type 1 spinal muscular atrophy (SMA), the most severe, infantile onset form of this rare and devastating neuromuscular disease. Risdiplam has been well tolerated and no treatment-related safety findings leading to withdrawal have been observed in any risdiplam trial to date. Data from part 2 of the FIREFISH study will be shared with health authorities globally and will be presented at an upcoming medical congress.

"We are excited about the FIREFISH results as they demonstrate how effective risdiplam is in type 1 SMA patients, where developmental milestones such as rolling, sitting and standing were achieved," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "These results further support the growing body of evidence of risdiplam's benefit in SMA patients across all types studied and reinforce the potential of our small molecule splicing platform to identify new therapies for patients who have limited or no treatment options."

The single-arm part 2 of FIREFISH study assessed the efficacy of risdiplam in 41 infants (eligible age at enrollment between 1 and 7 months) with type 1 SMA treated for 12 months. The primary endpoint in the study was defined as proportion of infants who are sitting without support after 12 months of treatment as assessed by the Bayley gross motor scale of infant and toddler development - third edition (BSID-III). Safety for risdiplam was consistent with its safety profile to date and no new safety signals were identified. No treatment-related safety findings leading to study withdrawal have been observed.

Risdiplam (RG7916), is an investigational, potential first-in-class oral mRNA splicing modifier for the treatment of SMA. FIREFISH is an open-label, two-part pivotal clinical trial in infants with type 1 SMA. Part 1 was a dose-finding study in 21 infants. The primary objectives of part 1 were to evaluate the safety profile of risdiplam in infants and to determine the dose for part 2. Part 1 of the trial showed that infants with type 1 SMA survived and achieved developmental milestones beyond those expected in the natural course of the disease. The SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

Recently, positive results from part 2 of SUNFISH, a study evaluating the efficacy and safety of risdiplam in patients between 2 and 25 years of age with type 2 or 3 SMA, were announced. In November 2019, the U.S. Food and Drug Administration (FDA) accepted a New Drug Application (NDA) and granted Priority Review for risdiplam. The NDA filing was based on 12-month data from the dose-finding part 1 of the pivotal FIREFISH and SUNFISH studies, and preclinical pharmacokinetic and clinical and pharmacodynamic data in all types of SMA. The Prescription Drug User Fee Act (PDUFA) goal date for a decision by FDA is May 24, 2020.

About Spinal Muscular Atrophy (SMA)
Spinal muscular atrophy (SMA) is a severe, inherited, progressive neuromuscular disease that causes devastating muscle atrophy and disease-related complications. It is the most common genetic cause of infant mortality and one of the most common rare diseases, affecting approximately one in 11,000 babies. SMA leads to the progressive loss of nerve cells in the spinal cord that control muscle movement. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

SMA is caused by a mutation in the survival motor neuron 1 (SMN1) gene that results in a deficiency of SMN protein. SMN protein is found throughout the body and increasing evidence suggests SMA is a multi-system disorder and the loss of SMN protein may affect many tissues and cells, which can stop the body from functioning.

About risdiplam
Risdiplam is an investigational survival motor neuron2 (SMN2) splicing modifier for SMA and is an orally administered liquid. It is designed to durably increase and sustain SMN protein levels both throughout the central nervous system and in peripheral tissues of the body.

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.

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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: the future expectations, plans and prospects for PTC; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, regulatory approvals or royalty or milestone payments; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "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 results of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any potential regulatory submissions and potential commercialization with regards to risdiplam; the eligible patient base and commercial potential of risdiplam or any of PTC's other product candidates; 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. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including risdiplam.

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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