



PTC Therapeutics Announces FDA Grants Priority Review to Risdiplam for the Treatment of Spinal Muscular Atrophy

November 25, 2019

- PDUFA date set for May 24, 2020-

- NDA filing is based on data in a broad SMA population of Type 1, 2 and 3 patients-

- FDA filing triggers \$15M milestone payment to PTC from Roche-

SOUTH PLAINFIELD, N.J., Nov. 25, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the United States Food and Drug Administration (FDA) has granted priority review for the New Drug Application (NDA) for risdiplam (RG7916) for the treatment of spinal muscular atrophy (SMA). The Prescription Drug User Fee Act (PDUFA) goal date for a decision by the FDA is May 24, 2020. The filing acceptance by the FDA triggers a \$15M milestone payable to PTC by Roche.

"The FDA's acceptance of the NDA is an important step towards making risdiplam available to SMA patients in the U.S.," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We are proud that risdiplam, the first oral small molecule targeting splicing, was produced from our proprietary splicing platform. Risdiplam's NDA submission includes results from a broad SMA patient population, including type 1, type 2 and type 3 SMA patients demonstrating improvements in motor functions and developmental milestones, and a compelling safety profile. We believe that an oral therapeutic that reaches all affected tissues in the body would mark a significant advancement in the treatment for SMA patients and their families."

The FDA has granted risdiplam priority review status, which is designated to drugs that, if approved, would represent significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.

The NDA filing is based on 12-month data from the dose-finding portion of the pivotal FIREFISH and SUNFISH studies, and clinical and preclinical pharmacokinetic and pharmacodynamic data. FIREFISH is an open-label, two-part clinical trial of risdiplam in infants with SMA type 1. SUNFISH is a double-blind, two-part, placebo-controlled trial of risdiplam in patients with type 2 or 3 SMA aged 2-25 years. SUNFISH part 2 recently met its primary endpoint of change from baseline in the Motor Function Measure 32 scale. Results from the study will be presented at an upcoming medical congress. 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 deletion or mutation in the survival of motor neuron 1 (SMN1) gene, which results in reduced levels of SMN protein. The related SMN2 pre-mRNA is alternatively spliced producing only small amounts of functional SMN protein. Insufficient levels of SMN protein result in the progressive loss of motor neurons leading to muscle atrophy and death in its most severe form. It is estimated that 1 in every 11,000 newborn children will develop SMA.

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 being studied in a clinical trial for patients with type 1 SMA, called FIREFISH, in pre-symptomatic babies, RAINBOWFISH, in patients who have been in previous clinical trials for SMA, JEWELFISH and in SUNFISH, a placebo-controlled study in people aged 2-25 years with Type 2 or 3 SMA.

About the SMA collaboration

The SMA program was initiated 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 U.S. 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-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 the NDA for risdiplam and any other potential regulatory submissions 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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