PTC Announces Regulatory Update on Risdiplam for Spinal Muscular Atrophy (SMA)

April 7, 2020

- U.S. Food and Drug Administration (FDA) extends review of risdiplam by three months following submission of additional data -

- Risdiplam has been studied across a broad real-world population of infants, children, teenagers and adults with Type 1, 2 or 3 SMA -

- European Medicine Agency (EMA) submission remains on track for mid-2020 -

SOUTH PLAINFIELD, N.J., April 7, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) date for its review of the New Drug Application (NDA) of risdiplam to August 24, 2020. Roche recently submitted additional data including comprehensive data from SUNFISH part 2 to help provide access to risdiplam for a broad range of people living with spinal muscular atrophy (SMA), triggering this extension. The FDA has informed Roche that the review team is working expeditiously to complete their review of the application as quickly as possible. The FDA has also indicated to Roche that no substantive review issues have been identified to date.

"We are encouraged that the FDA has no substantive review issues. Their interest in the additional results from the clinical studies demonstrating risdiplam's activity supports our goal of enabling access to this important therapy for all SMA patients," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "Enabling access to a home-administered oral therapy for a broad patient population is critically important and we look forward to the FDA living up to its commitment to review the application as quickly as possible."

In order to further support broad, global access to risdiplam for people living with SMA, Roche has submitted applications for approval in Indonesia, Taiwan, Chile, Brazil, South Korea and Russia and the submission of the filing in China is imminent. The submission of a Marketing Authorization Application (MAA) to the EMA and filings in other international markets remain on track for mid-2020.

In November 2019, the FDA granted Priority Review for risdiplam. Risdiplam has the broadest clinical trial program being evaluated to treat SMA, with patients ranging from birth to 60 years old, and includes patients previously treated with SMA-targeting therapies, including approved treatments. The clinical trial population represents the diverse, real-world spectrum of people living with this disease with the aim of ensuring access for all appropriate patients. To date, more than 400 patients have been treated with risdiplam across all studies to date, with no treatment-related safety findings leading to study withdrawal in any risdiplam trial. The SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

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. Risdiplam is being studied in a broad clinical trial program in SMA, with patients ranging from birth to 60 years old, and includes patients previously treated with other SMA-targeting therapies. The clinical trial population represents the broad, real-world spectrum of people living with this disease. The risdiplam clinical development program was designed with the aim of enabling access for all appropriate patients.

Risdiplam is currently being evaluated in four multicenter trials in people with SMA:

- SUNFISH (NCT02908685) – SUNFISH is a two-part, double-blind, placebo-controlled pivotal study in people aged 2-25 years with types 2 or 3 SMA. Part 1 (n=51) determined the dose for the confirmatory Part 2. Part 2 (n=180) evaluated motor function using total score of Motor Function Measure 32 (MFM-32) at 12 months. MFM-32 is a validated scale used to evaluate fine and gross motor function in people with neurological disorders, including SMA.
- FIREFISH (NCT02913482) – an open-label, two-part pivotal clinical trial in infants with type 1 SMA. Part 1 was a dose-escalation study in 21 infants. The primary objective of Part 1 was to assess the safety profile of risdiplam in infants and determine the dose for Part 2. Part 2 is a pivotal, single-arm study of risdiplam in 41 infants with type 1 SMA treated for 24 months, followed by an open-label extension. Enrollment for Part 2 was completed in November 2018. The primary objective of Part 2 is to assess efficacy as measured by the proportion of infants sitting without support after 12 months of treatment, as assessed in the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development – Third Edition (BSID-III) (defined as sitting without support for 5 seconds).
- JEWELFISH (NCT03032172) – an open-label exploratory trial in people with SMA aged 6 months–60 years who have

been previously treated with SMA-directed therapies. The study has completed recruitment.
- RAINBOWFISH (NCT03779334) – an open-label, single-arm, multicenter study, investigating the efficacy, safety, pharmacokinetics and pharmacodynamics of risdiplam in babies (~n=25), from birth to six weeks of age (at first dose) with genetically diagnosed SMA who are not yet presenting with symptoms. The study is currently recruiting.

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 and diversified 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 at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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 commercial prospects; 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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