Evrysdi™ (risdiplam) Continues to Make Global Progress for the Treatment of Spinal Muscular Atrophy

October 21, 2020

- Broad approval in Brazil based on data from multiple pivotal trials -

- NDA filed with Japan's MHLW under Priority Review; Milestone triggers $7.5M payment from Roche -

SOUTH PLAINFIELD, N.J., Oct. 21, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Evrysdi™ (risdiplam) was approved in Brazil by the National Health Surveillance Agency (ANVISA) for the treatment of spinal muscular atrophy (SMA). The approval was obtained within seven months of Roche's initial submission and marks the second country to approve Evrysdi™. Additionally, PTC announced the filing of a New Drug Application (NDA) for Evrysdi™ for the treatment of SMA with the Japanese Ministry of Health, Labor and Welfare (MHLW). The filing in Japan by Chugai Pharmaceutical Co. Ltd., a member of the Roche group, triggers a $7.5 million milestone payment to PTC from Roche.

"We are very pleased with both the rapid approval in Brazil as well as the filing of Evrysdi™ in Japan," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "These important achievements continue to build momentum for Evrysdi™ globally, and we are working hard alongside our partners in the collaboration to make the medicine available to SMA patients around the world. The strong efficacy, excellent safety profile, and convenient oral formulation of Evrysdi™ are so important to the SMA community. Access to a home administered therapy is particularly important during the COVID-19 pandemic."

The NDA in Japan is based on data from the FIREFISH study in infants aged 1 to 7 months old with symptomatic Type 1 SMA and the SUNFISH study in children and young adults with Type 2 or 3 SMA. MHLW previously granted orphan drug designation to risdiplam and the NDA application is subject to priority review.

The U.S. Food and Drug Administration (FDA) approved Evrysdi™ (risdiplam) in August 2020, for the treatment of SMA for adults and children 2 months and older. At this time, applications have been filed for Evrysdi™ in 15 international markets: Australia, Chile, India, Indonesia, Israel, Kuwait, Macedonia, Malaysia, Russia, Singapore, South Korea, Taiwan, Thailand, Ukraine and the United Arab Emirates. In addition, four health authorities worldwide are currently reviewing the application: Canada, China, the European Union and Switzerland.

Evrysdi™ is based on PTC science and is commercialized in the United States by Genentech, a member of the Roche Group. Roche leads the clinical development of Evrysdi™ as part of a collaboration with the SMA Foundation and PTC Therapeutics.

About Spinal Muscular Atrophy (SMA)
Spinal muscular atrophy (SMA) is a severe, progressive neuromuscular disease that can be fatal. It affects approximately 1 in 10,000 babies and is the leading genetic cause of infant mortality. SMA is caused by a mutation of the survival motor neuron 1 (SMN1) gene, which leads to a deficiency of SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Without it, nerve cells cannot function correctly, leading to muscle weakness over time. 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.

About Evrysdi™ (risdiplam)
Evrysdi™ is a survival motor neuron 2 (SMN2)-directed RNA splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi™ is designed to distribute evenly to all parts of the body, including the central nervous system (CNS). Evrysdi™ is administered daily at home in liquid form by mouth or feeding tube. The U.S. Food and Drug Administration recently approved Evrysdi™ (risdiplam) for the treatment of spinal muscular atrophy for adults and children 2 months and older. Evrysdi™ (risdiplam) is marketed in the United States by Genentech, a member of the Roche Group.

About the Risdiplam Clinical Studies

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 with the primary objective of assessing the safety profile of risdiplam in infants and determining the dose for Part 2. Part 2 is a pivotal, single-arm study of risdiplam in 41 infants with Type 1 SMA treated for two years followed by an open-label extension. Enrollment for Part 2 was completed in November 2018. The primary objective of Part 2 was 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 five seconds). The study met its primary endpoint.

SUNFISH (NCT02908685) - SUNFISH is a two part, double-blind, placebo controlled pivotal study in people aged 2 to 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. The study met its primary endpoint.

Clinical Trial Safety Data

The safety profile of Evrysdi™ was established across FIREFISH and SUNFISH pivotal trials. The most common adverse reactions in later-onset SMA (incidence of at least 10 percent of patients treated with Evrysdi™ and more frequently than control) were fever, diarrhea, and rash. The most common adverse reactions in infantile-onset SMA were similar to those observed in later-onset SMA patients. Additionally, the most common adverse reactions (incidence of at least 10 percent) were upper respiratory tract infection, pneumonia, constipation, and vomiting.

In addition to FIREFISH and SUNFISH, Evrysdi™ is being evaluated in a broad range of people with SMA, including in:

JEWELFISH (NCT03032172) - an open-label exploratory trial designed to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) in people with SMA aged 6 months to 60 years who received other investigational or approved SMA therapies for at least 90 days prior to receiving Evrysdi™. The study has completed recruitment (n=174).

RAINBOWFISH (NCT03779334) - an open-label, single-arm, multi-center study, investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of risdiplam in babies (~n=25), from birth to 6 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.

For More Information:

Investors:
Lisa Hayes
+1 (732) 354-8687
lhayes@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

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 historical 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 and, 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 outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future, including Evrysdi™; 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 Evrysdi™; the eligible patient base and commercial potential of Evrysdi™ or any of PTC's other product candidates; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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 (Securities and Exchange Commission). 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 or product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Evrysdi™.

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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