PTC Announces the Acceptance of the European Marketing Authorization Application for Evrysdi™ (risdiplam) for the Treatment of Spinal Muscular Atrophy

August 17, 2020

- Marketing Authorization Application (MAA) based on data from multiple pivotal trials in infants, children and adult patients with SMA -

- Milestone triggers $15M payment from Roche -

SOUTH PLAINFIELD, N.J., Aug. 17, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the acceptance of the Marketing Authorization Application (MAA) by the European Medicines Agency (EMA) for Evrysdi™ (risdiplam) for the treatment of spinal muscular atrophy (SMA). The EMA previously granted PRIME (PRIority MEdicines) designation to risdiplam for the treatment of people with SMA, providing a pathway for accelerated evaluation by the agency. The milestone triggers a $15 million payment to PTC from Roche.

"The acceptance of the MAA for Evrysdi™ marks an important milestone as we continue towards the goal of making this ground-breaking therapy available globally to a broad range of SMA patients," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Evrysdi™ has consistently demonstrated clinically meaningful results in multiple clinical trials with a favorable safety profile. Evrysdi™ is an oral medicine that can be delivered and taken at-home, which is particularly important for SMA patients during the COVID-19 global pandemic."

The submission, which was filed by Roche, is based on data from the dose-finding Part 1 and confirmatory Part 2 of the FIREFISH and SUNFISH studies which evaluated the efficacy and safety of Evrysdi™ (risdiplam) in symptomatic infants with type 1 SMA aged 2 to 7 months and in people with types 2 or 3 SMA aged 2 to 25 years, respectively. In addition, the submission incorporates safety data from JEWELFISH, a trial in people with all types of SMA aged 1 to 60 years previously treated with other SMA therapies. The results of an exploratory efficacy analysis from SUNFISH Part 1 showed Evrysdi™ (risdiplam) significantly improved motor function after 24 months of treatment compared to natural history data. Part 2 of the FIREFISH study met its primary endpoint of infants sitting without support for five seconds by month 12, as assessed by the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development Third Edition (BSID-III). Part 2 of the SUNFISH trial in non-ambulatory children and adults demonstrated that change from baseline in total Motor Function Measure 32 (MFM-32) score was significantly greater at 12 months in people treated with Evrysdi™ (risdiplam), compared to placebo.

The U.S. Food and Drug Administration recently approved Evrysdi™ (risdiplam) for the treatment of SMA for adults and children 2 months and older. Evrysdi™ (risdiplam) is based on PTC science and is commercialized in the United States by Genentech, a member of the Roche Group. Evrysdi™ is a product of the SMA 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 loss of function mutations or deletions of 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.

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 study, was designed to assess Evrysdi™ safety, tolerability, efficacy, pharmacokinetics (PK) and pharmacodynamics (PD) in patients aged 1 to 7 months with type 1 SMA. Part 1 evaluated several doses of Evrysdi™ and determined the therapeutic dose for Part 2.

SUNFISH (NCT02908685): a two-part, placebo-controlled, multicenter pivotal trial, was designed to assess Evrysdi™ safety, tolerability, efficacy, PK and PD in people with type 2 or 3 SMA aged 2 to 25, including those with scoliosis and joint contractures at baseline.

Clinical Trial Safety Data

The safety profile of Evrysdi™ (risdiplam) was established across FIREFISH and SUNFISH pivotal trials. The most common adverse reactions in later-onset SMA (incidence of at least 10% 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%) 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, PK and 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™. Recruitment for this study is complete with 174 people enrolled.

RAINBOWFISH (NCT03779334): an open-label, single-arm, multicenter study investigating the efficacy, safety, PK and PD of Evrysdi™ in infants (~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.

For More Information:
Investors & 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. 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.

View original content:http://www.prnewswire.com/news-releases/ptc-announces-the-acceptance-of-the-european-marketing-authorization-application-for-evrysdi-risdiplam-for-the-treatment-of-spinal-muscular-atrophy-301113095.html

SOURCE PTC Therapeutics, Inc.