Results from the Second Year of Evrysdi™ (risdiplam) Treatment Demonstrated Sustained Improvement of Motor Function in a Broad Range of SMA Patients

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- Sustained or improved motor function was observed with 24 months of treatment -

- Patients and caregivers reported a greater ability to complete activities of daily livingwith increased independence -

SOUTH PLAINFIELD, N.J., March 16, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced two-year data from Part 2 of the SUNFISH trial evaluating Evrysdi™ (risdiplam) in children and adults with Type 2 or Type 3 spinal muscular atrophy (SMA) at the 2021Muscular Dystrophy Association (MDA) Virtual and Scientific Conference. These results demonstrated that Evrysdi patients sustained or improved in motor function after 24 months of treatment. Furthermore, the patients and caregivers reported improvements in their ability to function independently as well as their ability to complete daily tasks.

"We're encouraged by the long-term results from the SUNFISH trial, which reinforce the sustained clinical benefit that Evrysdi offers to a very broad population of patients with SMA, many of whom experience difficulty performing daily functions," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "These data further support the strong U.S. launch and the need for an at home oral treatment option for the SMA community."

Two-year data from SUNFISH Part 2 include efficacy and safety data in the overall population (2-25 years old):

- MFM32 (Motor Function Measure – 32), RULM (Revised Upper Limb Module) and HFMSE (Hammersmith Functional Motor Scale Expanded) scores showed improvement or maintenance of motor function during the second year of treatment with Evrysdi.
- Maintained motor function improvements between months 12 and 24 as measured by Motor Function Measure (MFM-32).
- Increased motor function as measured by Revised Upper Limb Module (RULM) and the Hammersmith Functional Motor Scale-Expanded (HFMSE) between months 12 and 24.
- Stabilized motor function for patients who began treatment with Evrysdi after 12 months of placebo as measured by MFM-32, RULM and HFMSE.
- Increased total score change from baseline, as measured by the caregiver-reported SMAIS (SMA Independence Scale) upper limb module, and the patient-reported SMAIS score stabilized between months 12 and 24.

Decreases in serious adverse events, high-grade adverse events and treatment-related adverse events were observed in the second year versus the first year in both treatment arms. The most common adverse events observed in the Evrysdi arm and the placebo and Evrysdi arm from 12-24 months were upper respiratory tract infection (15.8% and 10%, respectively), nasopharyngitis (21.7% and 16.7%, respectively), pyrexia (13.3% and 10%, respectively), headache (10% and 16.7%, respectively), diarrhea (7.5% and 10%, respectively), vomiting (11.7% and 13.3%, respectively) and cough (10% and 8.3%, respectively). The most common serious adverse events were pneumonia (6.7% and 0%, respectively) and influenza (0.8% and 0%, respectively).

Evrysdi is designed to treat SMA by increasing and sustaining the production of the SMN protein, which is found throughout the body and is critical for maintaining healthy motor neurons and movement. Roche leads the clinical development of Evrysdi as part of a collaboration with the SMA Foundation and PTC Therapeutics. Evrysdi is marketed in the United States by Genentech, a member of the Roche Group.

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 throughout the central nervous system (CNS). Evrysdi is administered daily at home in liquid form by mouth or feeding tube. In August 2020, the U.S. Food and Drug Administration approved Evrysdi for the treatment of spinal muscular atrophy for adults and children 2 months and older. Evrysdi is marketed in the United States by Genentech, a member of the Roche Group. Evrysdi is also approved in seven countries and has been filed in more than 50 countries worldwide.

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.

About the Evrysdi 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) - 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.

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 Spinal Muscular Atrophy (SMA)
Spinal muscular atrophy (SMA) is a severe, progressive neuromuscular disease that can be fatal. It affects approximately one in 10,000 babies and left untreated 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 PTC

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. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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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, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; advancement of PTC's joint collaboration program in SMA, including any regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; 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 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; the enrollment, conduct, and results of ongoing 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 regulatory submissions and commercialization with respect to Evrysdi; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; 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 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/results-from-the-second-year-of-evrysdi-risdiplam-treatment-demonstrated-sustained-improvement-of-motor-function-in-a-broad-range-of-sma-patients-301247815.html

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