FDA Grants Evrysdi® Priority Review Based on Results From Treating Pre-Symptomatic Infants with Spinal Muscular Atrophy

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- RAINBOWFISH study interim results showed that after treatment with Evrysdi pre-symptomatic infants with spinal muscular atrophy achieved same motor milestones as healthy infants -

SOUTH PLAINFIELD, N.J., Jan. 25, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the U.S. Food and Drug Administration (FDA) has granted priority review of a supplemental new drug application (sNDA) for Evrysdi® (risdiplam) to expand the indication to include pre-symptomatic infants under 2 months old with spinal muscular atrophy (SMA). If approved, Evrysdi would be the first medicine administered at-home for pre-symptomatic babies with SMA.

"The results demonstrating that almost all of the pre-symptomatic infants achieved motor milestones comparable to healthy infants is tremendous," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "The granting of the Priority Review for Evrysdi recognizes this and the significant need to treat babies with SMA as early as possible. We are proud that such a transformative treatment for patients living with SMA came from our splicing platform."

Included in the sNDA submission was interim data from the RAINBOWFISH study, which showed 80 percent of pre-symptomatic infants with SMA treated with Evrysdi for at least 12 months achieved motor milestones such as sitting without support, rolling, crawling, standing unaided, and walking independently.

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. Evrysdi was based on PTC's splicing platform. Evrysdi is marketed by Roche and 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 when 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 progressive 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), and it is administered daily at home in liquid form by mouth or feeding tube. The FDA recently 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, and marketed in rest of world by Roche.

About the Evrysdi® Clinical Studies

FIREFISH (NCT02913482) is 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 Evrysdi in infants and determining the dose for Part 2. Part 2 is a pivotal, single-arm study of Evrysdi in 41 infants with Type 1 SMA treated for two years followed by an open-label extension. 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) 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 the Motor Function Measure 32 (MFM-32) scale 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 in the following studies:

JEWELFISH (NCT03032172) is 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) is an open-label, single-arm, multi-center study, investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of Evrysdi in babies (~n=25) from birth to 6 weeks old (at first dose), with genetically diagnosed SMA, who are not yet presenting symptoms. The study is currently recruiting.

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and 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: advancement of PTC's joint collaboration program in SMA, including the commercialization of any products therein or royalty or milestone payments; 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, licensing or commercialization of its products and products candidates and other matters; 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.

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