



## Significant Improvements in Survival and Developmental Milestones Demonstrated in Type 1 SMA Patients Treated with Evrysdi™

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### - Results from Part 2 of FIREFISH pivotal study published in the New England Journal of Medicine -

SOUTH PLAINFIELD, N.J., July 28, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that results from Part 2 of the FIREFISH pivotal study were published in the New England Journal of Medicine. This trial showed that symptomatic Type 1 spinal muscular atrophy (SMA) infants treated with Evrysdi™ (risdiplam) showed significant improvements in survival, motor milestones, and motor function compared to historical controls.

"It is exciting to see such profound results in infants treated with Evrysdi," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "We are proud that such a significant treatment for SMA patients was developed from PTC's splicing platform. We believe that the splicing platform is a disruptive technology that will continue to drive the development of new therapies in diseases with no treatment options available."

The table below depicts the primary and secondary endpoints achieved across Part 2 of the FIREFISH study after 12 months of treatment:

Milestone Achievement	Percentage of Infants Achieving Milestones After 12 months with Evrysdi	Historical Controls
Ability to sit without support for at least five seconds	29% (12/41)	5%
CHOP-INTEND* score of 40 or more	56% (23/41)	17%
An increase of at least 4 points in the CHOP-INTEND total score from baseline	90% (37/41)	17%
HINE-2** motor-milestone responders	78% (32/41)	12%
Event free survival‡	85% (35/41)	42%

FIREFISH is an open-label, two-part, pivotal clinical trial designed to evaluate the efficacy, safety, tolerability, pharmacokinetics and pharmacodynamics in infants with type 1 SMA. In the dose-finding Part 1 of the study, treatment with Evrysdi led to an increased expression of SMN protein levels. Part 2 evaluated the efficacy and safety of Evrysdi in Type 1 SMA.

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.

\* CHOP-INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders

\*\* HINE-2, Hammersmith Infant Neurological Examination

‡ Event-free survival is defined as alive without need for permanent ventilation (defined as requiring tracheostomy or ventilation for ≥16 hours per day continuously for >3 weeks or continuous intubation >3 weeks, in the absence of, or following the resolution of, an acute reversible event). Data cut-off: 14 Nov 2019.

### 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). Evrysdi™ is administered daily at home in liquid form by mouth or feeding tube. Evrysdi has

been approved in over 50 countries including the United States, the European Union and Japan. Evrysdi™ (risdiplam) is marketed in the United States by Genentech, a member of the Roche Group.

### **About the Evrysdi™ (risdiplam) 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 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. 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.

**RAINBOWFISH (NCT03779334)** is 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 old (at first dose), with genetically diagnosed SMA, who are not yet presenting symptoms. The study is currently recruiting.

**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).

### **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 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](http://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: 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.

 View original content: <https://www.prnewswire.com/news-releases/significant-improvements-in-survival-and-developmental-milestones-demonstrated-in-type-1-sma-patients-treated-with-evrysdi-301343499.html>

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