Media Release

Roche's Evrysdi tablet approved by European Commission as first and only for Spinal Muscular Atrophy (SMA)

- **Simplified storage and administration of new tablet formulation may provide greater freedom and independence for people with SMA**
- **Evrysdi offers the same efficacy and safety demonstrated in available oral solution**
- **Evrysdi is the only non-invasive disease-modifying SMA treatment, with more than 18,000 people with SMA treated globally to date**

Basel, 04 June 2025 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the European Commission (EC) has approved a label extension for Evrysdi® (risdiplam) to include a new, room-temperature *stable* tablet for people living with spinal muscular atrophy (SMA). The 5mg tablet (approx. 6.5mm), which can either be swallowed whole or dispersed in water, can be taken with or without food and does not require refrigeration, when stored at room temperature. Administered at home, Evrysdi is the only non-invasive disease modifying treatment available for people living with SMA.

"The new Evrysdi tablet with its flexible administration represents progress toward more versatile SMA disease management," said Levi Garraway, M.D., Ph.D., Chief Medical Officer and Head of Global Product Development, Roche. "With over 18,000 people treated to date, Evrysdi's proven efficacy, safety and convenience has significantly improved the course of disease for people living with SMA."

Evrysdi is designed to treat SMA by increasing and sustaining the production of SMN protein throughout the entire central nervous system (CNS) and in peripheral tissues. Together with this innovative mode of action, the new tablet formulation offers additional portability and convenience benefits for the thousands of people living with SMA, their families and caregivers.

"We welcome the development of new treatment formulations that have the potential to further simplify disease management and care for people living with SMA," Nicole Gusset, Chief Executive Officer, SMA Europe commented. "This is a disease requiring daily management, and it is paramount that people living with SMA, and those who care for them, are given options to optimise treatment administration."

The approval is based on data from a bioequivalence study (NCT04718181) evaluating the 5mg tablet formulation of Evrysdi, which can either be swallowed whole or dispersed in water. Results presented at SMA Europe's 4th Scientific International Congress in 2024 demonstrated that the tablet formulation and original oral solution provided bioequivalence

to Evrysdi, meaning individuals taking the tablet can expect the same established efficacy and safety as the oral solution.

The 5mg tablet formulation is suitable for people two years of age or older, who weigh 20kg (44 lbs) or more and are able to swallow without the use of a feeding tube. The original oral solution will remain available for those on other doses of Evrysdi and for those who may prefer the oral solution.

Roche leads the clinical development of Evrysdi as part of a collaboration with the SMA Foundation and PTC Therapeutics.

About Evrysdi® (risdiplam)

Evrysdi is a survival motor neuron 2 (SMN2) splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to survival of motor neuron (SMN) protein deficiency. Evrysdi is administered daily at home or on the go, either in liquid form (by feeding tube or by mouth) or in the form of a tablet, which can either be swallowed whole or dispersed in water.

Evrysdi is designed to treat SMA by increasing and sustaining the production of SMN protein in the CNS and peripheral tissues. SMN protein is found throughout the body and is critical for maintaining healthy motor neurons and core functions.

Evrysdi was granted PRIME designation by the European Medicines Agency (EMA) in 2018 and Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) in 2017. In 2021, Evrysdi was awarded Drug Discovery of the Year by the British Pharmacological Society as well as the Society for Medicines Research Award for Drug Discovery. Evrysdi is currently approved in more than 100 countries, with more than 18,000 people with SMA treated globally.

Evrysdi is currently being, or has been, evaluated in numerous global multicenter trials in people with SMA:

- FIREFISH (NCT02913482) – an open-label, two-part pivotal clinical trial in infants with Type 1 SMA. Infants were approximately 5.5 months of age (median) at the time of enrollment and of the 58 infants that completed the first year of treatment, 52 entered the open-label extension study. The study met its primary endpoint and has concluded after 5 years of follow up.
- SUNFISH (NCT02908685) – a two-part, double-blind, placebo-controlled pivotal study in people aged 2-25 years with Types 2 or 3 SMA. The study met its primary endpoint and has concluded after five years of follow up.
- JEWELFISH (NCT03032172) – an open-label exploratory trial designed to assess the safety, tolerability, pharmacokinetics and pharmacodynamics in people with SMA

aged 6 months to 60 years who received other investigational or approved SMA therapies prior to receiving Evrysdi. The study has completed recruitment (n=174).

- RAINBOWFISH (NCT03779334) – an open-label, single-arm, multicenter study, investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of Evrysdi in babies (n=26), from birth to 6 weeks of age (at first dose) with genetically diagnosed SMA who are not yet presenting with symptoms. The study met its primary endpoint.
- MANATEE (NCT05115110) – a Phase II/III clinical study to evaluate the safety and efficacy of GYM329 (RG6237), an anti-myostatin molecule targeting muscle growth, in combination with Evrysdi for the treatment of SMA in patients 2-10 years of age. The FDA Office of Orphan Products Development granted GYM329 Orphan Drug Designation for the treatment of patients with SMA in December 2021. The study is currently recruiting.
- HINALEA 1 (NCT05861986) and HINALEA 2 (NCT05861999) – Phase IV clinical studies to evaluate the effectiveness and safety of Evrysdi in patients under 2 years of age at enrollment, who received onasemnogene abeparvovec gene therapy either pre-symptomatically or post-symptomatically, following a genetically confirmed diagnosis of 5q–autosomal recessive SMA. The studies are currently recruiting.
- PUPFISH (NCT05808764) – a Phase II, open-label study to investigate the pharmacokinetics and safety of Evrysdi in babies with SMA who are under 20 days of age (at first dose). The study is currently recruiting.

About SMA

SMA is a severe, progressive neuromuscular disease that can be fatal. It affects approximately one 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 Roche in Neuroscience

Neuroscience is a major focus of research and development at Roche. Our goal is to pursue groundbreaking science to develop new treatments that help improve the lives of people with chronic and potentially devastating diseases.

Roche is investigating more than a dozen medicines for neurological disorders, including multiple sclerosis, spinal muscular atrophy, neuromyelitis optica spectrum disorder, Alzheimer's disease, Huntington's disease, Parkinson's disease and Duchenne muscular

dystrophy. Together with our partners, we are committed to pushing the boundaries of scientific understanding to solve some of the most difficult challenges in neuroscience today.

About Roche

Founded in 1896 in Basel, Switzerland, as one of the first industrial manufacturers of branded medicines, Roche has grown into the world's largest biotechnology company and the global leader in in-vitro diagnostics. The company pursues scientific excellence to discover and develop medicines and diagnostics for improving and saving the lives of people around the world. We are a pioneer in personalised healthcare and want to further transform how healthcare is delivered to have an even greater impact. To provide the best care for each person we partner with many stakeholders and combine our strengths in Diagnostics and Pharma with data insights from the clinical practice.

For over 125 years, sustainability has been an integral part of Roche's business. As a science-driven company, our greatest contribution to society is developing innovative medicines and diagnostics that help people live healthier lives. Roche is committed to the Science Based Targets initiative and the Sustainable Markets Initiative to achieve net zero by 2045.

Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan.

For more information, please visit www.roche.com.

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