Roche presents new data at World Muscle Society (WMS) 2021 highlighting new advances for people living with rare neuromuscular disorders

- **New data show pre-symptomatic babies with spinal muscular atrophy (SMA) treated with Evrysdi maintained the ability to swallow**
- **Evrysdi has demonstrated consistent clinically meaningful efficacy in adults, children, and babies two months and older and is now approved in 58 countries worldwide**
- **Further presentations included data from studies supporting the efficacy, safety, and durability of gene therapy, SRP-9001, in the treatment of Duchenne muscular dystrophy (DMD)**

Basel, 24 September 2021 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced data from across its growing neuromuscular portfolio at the World Muscle Society (WMS) Virtual Congress 20 – 24 September 2021. The presentations included additional results from the RAINBOWFISH study, evaluating the efficacy and safety of Evrysdi® (risdiplam) in babies with pre-symptomatic spinal muscular atrophy (SMA) from birth to six weeks of age and data supporting the continued clinical investigation of gene therapy, SRP-9001, in Duchenne muscular dystrophy (DMD).

"These new data for Evrysdi may help extend the potential benefits of this medicine to the youngest SMA patients. Also, the data from SRP-9001 have helped to optimise the design of the upcoming Phase III trial for DMD," said Levi Garraway, M.D., Ph.D, Roche's Chief Medical Officer and Head of Global Product Development. "Our goal is to continue to lead the way in developing transformative medicines for neuromuscular diseases. We are grateful for the partnerships that are helping us to develop new therapies for people impacted by these devastating rare diseases."

At WMS 2021, data from the ongoing open label RAINBOWFISH study were presented. Four out of five of those treated with Evrysdi for at least 12 months achieved standing and walking independently within the World Health Organization windows for healthy children. In addition, all five babies maintained the ability to swallow and were able to feed exclusively orally after 12 months of treatment.

Previously reported results showed that babies treated with Evrysdi for at least 12 months achieved Hammersmith Infant Neurological Examination (HINE-2) motor milestones, with 100% (n=5) able to maintain head control, sitting upright, rolling and crawling.

These data further add to the growing body of evidence supporting Evrysdi's efficacy in a broad patient population. More than 4,000 patients have been treated with Evrysdi in clinical trials, compassionate use, and real-world settings.

In DMD, three-year data from the open-label trial, Study SRP-9001-101, evaluating the safety of a single dose of the investigational gene therapy SRP-9001 in four ambulatory children aged 4 and 7 years old with DMD,

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were presented. The study showed that SRP-9001 was well-tolerated with key functional assessment, measured by the North Star Ambulatory Assessment (NSAA), demonstrating an overall improvement in motor ability compared to baseline. The improvements in motor abilities were maintained over three years, signifying a durable response. The results support the continuation of clinical investigation to further assess the benefit/risk of SRP-9001 in patients with DMD.

Roche, and partner Sarepta, also shared data from ENDEAVOR (Study SRP-9001-103), the first clinical trial using commercially representative SRP-9001 material for the treatment of DMD. Interim results from the first 11 participants in Cohort 1 (ambulatory boys aged 4-7 years) from the open label, ongoing Phase 1b study, provides evidence that SRP-9001 showed robust expression of micro-dystrophin and no new safety signals were identified.

In addition, results from Part 1 of Study SRP-9001-102, an ongoing, randomised, double-blind, placebo-controlled clinical trial evaluating the safety, efficacy, and tolerability of a single dose of SRP-9001 in 41 boys with DMD, showed that the study met its primary biological endpoint of change in micro-dystrophin protein expression from baseline. Participants treated with SRP-9001, generally showed an increase in NSAA total score compared to placebo at 48 weeks, although this increase did not achieve statistical significance compared to that of patients who received placebo. The safety profile was consistent with prior studies, with no new safety signals identified.

The results of these studies provide important information for SRP-9001's ongoing clinical development programme, with outcomes from Study 101 and 102 informing the design of the Phase 3 trial for SRP-9001, due to commence globally by the end of the year.

About Evrysdi® (risdiplam)
Evrysdi is a survival of motor neuron 2 (SMN2) splicing modifier designed to treat SMA by increasing production of the survival of motor neuron (SMN) protein. SMN protein is found throughout the body and is critical for maintaining healthy motor neurons and movement. Evrysdi is administered daily at home in liquid form by mouth or by feeding tube.

The U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) approved Evrysdi for the treatment of SMA in adults and children 2 months of age and older. Evrysdi was granted PRIME designation by the EMA in 2018 and Orphan Drug Designation by FDA and EMA in 2017 and 2019, respectively. At this time, Evrysdi has been approved in over 50 countries including the US and Europe and submitted in more than 30 countries.

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 SRP-9001
SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) is an investigational gene therapy designed to deliver the micro-dystrophin-encoding gene directly to the skeletal and cardiac muscle for the targeted production of the micro-dystrophin protein to enable a durable clinical response. Sarepta Therapeutics is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval. In December 2019, Roche partnered with Sarepta to combine Roche's global reach, commercial presence, and regulatory expertise to accelerate access to SRP-9001 for patients outside the United States.

About DMD
DMD is a rare X-linked, progressive neuromuscular disease caused by mutations in the DMD gene that disrupts the production of functional dystrophin protein, leading to a loss of muscle function and premature death. DMD is one of the most common fatal genetic disorders, affecting approximately one in every 3,500 to 5,000 male births worldwide.

Symptoms usually appear in infants and toddlers, with affected children presenting developmental delays such as difficulty walking, climbing stairs or standing from a sitting position. As DMD progresses, muscle weakness involves the arms, trunk, and other areas, meaning patients often require full-time use of a wheelchair in their early teens. Longevity is limited due to cardiac and/or respiratory failure.

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, neuromyelitis optica spectrum disorder, Alzheimer's disease, Huntington's disease, Parkinson's disease,

Duchenne muscular dystrophy and autism spectrum disorder. 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

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics, as well as growing capabilities in the area of data-driven medical insights help Roche deliver truly personalised healthcare. Roche is working with partners across the healthcare sector to provide the best care for each person.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. In recent years, Roche has invested in genomic profiling and real-world data partnerships and has become an industry-leading partner for medical insights.

Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. More than thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Moreover, for the twelfth consecutive year, Roche has been recognised as one of the most sustainable companies in the Pharmaceuticals Industry by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2020 employed more than 100,000 people worldwide. In 2020, Roche invested CHF 12.2 billion in R&D and posted sales of CHF 58.3 billion. 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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