Media & Investor Release

Roche to present new Evrysdi data at MDA 2022 and highlight expanding neuromuscular disease portfolio

- **Evrysdi® (risdiplam) data further confirm long-term efficacy and safety data in a broad range of people with spinal muscular atrophy (SMA)**
- **Latest interim results from the RAINBOWFISH study demonstrate that the majority of babies treated with Evrysdi for at least 12 months were able to sit, stand and walk within timeframes typical of healthy babies**
- **New gene therapy data in boys living with Duchenne muscular dystrophy (DMD) reports encouraging results from early stage study supporting its progress into global pivotal Phase III EMBARK study**

Basel, 08 March 2022 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that new data from its growing neuromuscular portfolio will be presented at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference, 13 – 16 March 2022. Presentations include eight abstracts from its spinal muscular atrophy (SMA) programme and three from its Duchenne muscular dystrophy (DMD) programme, demonstrating Roche's commitment to advancing clinical understanding of these conditions with the aim of treating a broad range of people living with neuromuscular disorders.

"These new data further highlight the compelling body of evidence for Evrysdi across infants, children and adults living with SMA," said Levi Garraway, M.D., Ph. D., Roche's Chief Medical Officer and Head of Global Product Development. "The results from our Duchenne Muscular Dystrophy gene therapy programme are encouraging and represent the potential to provide transformative outcomes for people living with this condition. We are grateful for the partnerships that can help us accelerate progress across SMA and DMD."

Spinal Muscular Atrophy (SMA)

Six abstracts from the Evrysdi® (risdiplam) clinical development programme will be presented. This includes 3-year SUNFISH Part 1 and 2 efficacy and safety data, as well as a comparison of 2-year SUNFISH Part 2 data with an untreated external control group, both of which highlight the long-term efficacy and safety profile of Evrysdi in a broad population of people aged 2-25 years with Type 2 or Type 3 SMA. The data also demonstrate sustained increase in motor function over time, compared to an untreated external control group at 2-years.

Updated interim data from the RAINBOWFISH study will report updated safety data in enrolled infants and efficacy data in infants who have received risdiplam for at least 12 months.

Roche will also be presenting data from four other SMA studies including:

- 24-month data from FIREFISH Part 1 and 2 in SMA Type 1, exploring the safety and efficacy of Evrysdi in infants treated with Evrysdi for 2-years
- 12-month data from the JEWELFISH study, exploring the safety, tolerability and pharmacokinetic/pharmacodynamic (PD) relationship of Evrysdi in a broad age range of patients (6 months to 60 years) who have previously received RG7800 (RO6885247), nursinersen, olesoxime or onasemnogene abeparvovec
- Design of new MANATEE trial, a multi-centre, randomised, placebo-controlled, double-blind study investigating the effect of GYM329, an investigational anti-myostatin, in combination with Evrysdi
- Data on the demographics and clinical characteristics of people with SMA enrolled in the Muscular Dystrophy Association Neuromuscular Observational Research (MOVR) hub
- Using baseline data from FIREFISH, SUNFISH, JEWELFISH and NAtHis-SMA studies, a validated algorithm for estimating the weight of patients with SMA based on few input parameters

Duchenne Muscular Dystrophy (DMD)

Roche has partnered with Sarepta to accelerate access to investigational gene therapy, delandistrogene moxeparvovec (SRP-9001), for DMD outside of the United States upon US Food and Drug Adminstration (FDA) approval. Sarepta, will present three year data from Study 101, an open-label Phase 1/2a study evaluating the safety of SRP-9001 in four ambulatory participants aged between 4-7 years old with DMD. The results show an acceptable safety profile, and all patients demonstrated a clinically meaningful improvement in their North Star Ambulatory Assessment (NSAA) from baseline.

In addition, Sarepta will also present results from Study 102 which continue to support SRP-9001's clinical profile and reinforce a potential benefit risk profile. Study 102 is a three-part, Phase 2 clinical study evaluating the safety and efficacy of delandistrogene moxeparvovec in patients with DMD. Analysis of Part 2 of the study will be presented.

Other presentations include:

- The study design and methodology of the first delandistrogene moxeparvovec global Phase III gene therapy trial, EMBARK, assessing the safety and efficacy of commercially representative delandistrogene moxeparvovec material in ambulatory boys with a confirmed DMD mutation aged 4 to 7 years old.

The full range of data from Roche's clinical development programme in neuroscience being presented at MDA 2022 include:

Medicine	**Abstract Title**	**Presentation Number (type), Presentation Date, Time**
Evrysdi® (risdiplam) for spinal muscular atrophy	SUNFISH Parts 1 and 2: 3-year efficacy and safety of risdiplam in Types 2 and 3 SMA	Data presentation Wednesday 16th March 8:20 – 8:35 am CT (14:20 – 14:35 CET)
	SUNFISH Part 2: 24-month efficacy of risdiplam compared with external control comparators	Posters Sunday 13th March 18:00 – 20:00 CT (02:00 – 04:00 am CET) Monday 14th March 10:00 am – 20:00 CT (16:00 – 02:00 am CET) Tuesday 15th March 10:00 am – 20:00 CT (16:00 – 02:00 am CET) Dedicated Poster sessions between 6:00-8:00pm daily
	MANATEE: A study of GYM329 (RO7204239) in combination with risdiplam treatment in pediatric patients with SMA	
	Demographics and clinical characteristics of individuals with spinal muscular atrophy enrolled in the Muscular Dystrophy Association MOVR data hub	
	FIREFISH Parts 1 and 2: 24-month safety and efficacy of risdiplam in infants with Type 1 SMA	
	JEWELFISH: Safety, pharmacodynamic and exploratory efficacy data in non-naïve patients with SMA receiving treatment with risdiplam	
	RAINBOWFISH: Preliminary efficacy and safety data in risdiplam-treated infants with presymptomatic SMA	
	Statistical modelling to estimate patients' weight in Types 1–3 SMA	
SRP-9001 for Duchenne muscular dystrophy	EMBARK study design: Phase 3 trial evaluating the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in Duchenne muscular dystrophy	
	Phase 1/2a trial of delandistrogene moxeparvovec (SRP-9001) in patients with Duchenne muscular dystrophy: 3-year safety and functional outcomes	
	A Phase 2 clinical trial evaluating the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in patients with Duchenne muscular dystrophy	

Full session details and data presentations listing for the MDA Clinical and Scientific Conference can be found at the meeting website: https://mdaconference.org/

Follow Roche on Twitter via @Roche and keep up to date with MDA 2022 Conference news and updates by using the hashtag #RocheAtMDA2022, #MDA and #neuromuscular

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 SMN protein deficiency. To date over 5,000 patients have been treated with Evrysdi, which is administered daily at home in liquid form by mouth or by feeding tube.

Evrysdi is designed to treat SMA by increasing and sustaining the production of the survival motor neuron (SMN) protein in the central nervous system (CNS) and peripheral tissues. SMN protein is found throughout the body and is critical for maintaining healthy motor neurons and movement.

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 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 75 countries and the dossier is under review in a further 27 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 delandistrogene moxeparvovec

Delandistrogene moxeparvovec (SRP-9001; rAAVrh74.MHCK7.micro-dystrophin) is an investigational gene therapy aimed to deliver the micro-dystrophin-encoding transgene 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 delandistrogene moxeparvovec and plans to commercialize delandistrogene moxeparvovec 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 delandistrogene moxeparvovec 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, spinal muscular atrophy, 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 in1896 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.

In recognizing our endeavor to pursue a long-term perspective in all we do, Roche has been named one of the most sustainable companies in the pharmaceuticals industry by the Dow Jones Sustainability Indices for the thirteenth consecutive year. This distinction also reflects our efforts to improve access to healthcare together with local partners in every country we work.

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.

Roche Group Media Relations
Phone: +41 61 688 8888 / e-mail: media.relations@roche.com

Dr. Nicolas Dunant
Phone: +41 61 687 05 17

Dr. Barbara von Schnurbein
Phone: +41 61 687 89 67

Nina Mählitz
Phone: +41 79 327 54 74

Sileia Urech
Phone: +41 79 935 81 48

Karsten Kleine
Phone: +41 61 682 28 31

Nathalie Meetz
Phone: +41 61 687 43 05

Roche Investor Relations

Dr. Karl Mahler
Phone: +41 61 68-78503
e-mail: karl.mahler@roche.com

Dr. Sabine Borngräber
Phone: +41 61 68-88027
e-mail: sabine.borngraeber@roche.com

Dr. Birgit Masjost
Phone: +41 61 68-84814
e-mail: birgit.masjost@roche.com

Dr. Bruno Eschli
Phone: +41 61 68-75284
e-mail: bruno.eschli@roche.com

Dr. Gerard Tobin
Phone: +41 61 68-72942
e-mail: gerard.tobin@roche.com

Investor Relations North America

Loren Kalm
Phone: +1 650 225 3217
e-mail: kalm.loren@gene.com