Media & Investor Release

Roche to share latest scientific advancements from its neuromuscular portfolio at Muscular Dystrophy Association (MDA) 2025 conference

- **New Evrysdi five-year data from the SUNFISH study showed continued stabilisation of motor function in a broad population of individuals with Types 2 or 3 spinal muscular atrophy (SMA)**
- **Late-breaking oral on Elevidys' Embark two-year data and pooled analysis of Study 101, 102 and Endeavor, demonstrated clinically meaningful and statistically significant improvements across key measures of motor function in boys with Duchenne muscular dystrophy (DMD)**
- **Muscle pathology as measured by MRI continued to generally favour Elevidys across muscle groups in boys with Duchenne up to two years**

Basel, 17 March 2025 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that it will present new data at the Muscular Dystrophy Association (MDA) conference, 16-19 March, 2025, in Dallas, Texas, from its neuromuscular portfolio, including 12 oral and poster presentations. These data demonstrate the breadth of Roche's neuromuscular portfolio across spinal muscular atrophy (SMA) and Duchenne muscular dystrophy (DMD).

"These longer-term results are encouraging for people living with SMA and Duchenne, as they demonstrate sustained improvements or stabilisation in mobility and independence for those receiving our disease-modifying treatments," said Levi Garraway, M.D., Ph.D., Chief Medical Officer and Head of Global Product Development. "The positive SUNFISH five-year results encompass one of the broadest SMA populations to be studied to date. We are also very encouraged by the data being shared across our Elevidys studies, affirming the potential of the first gene therapy for the treatment of Duchenne to stabilise or slow disease progression."

Five-year exploratory data from the pivotal SUNFISH study of Evrysdi in people with Types 2 or 3 SMA

After up to five years of treatment in the SUNFISH trial (NCT02908685, n=231), SMA patients on Evrysdi demonstrated overall long-term stabilisation of motor function improvements from baseline that were observed during the first year, as measured by Motor Function Measure 32 (MFM-32). This marks the final readout from SUNFISH, which has reinforced the efficacy, safety and longer-term impact of Evrysdi in a broad population of people with Types 2 and 3 SMA, aged 2-25 years at enrollment. Untreated natural history data presented together with the SUNFISH five-year data show that without treatment, patients from a

similar population can experience a significant decline in motor function over the same five-year period.

Patients (>12 years of age) and caregivers both reported continuous improvement or stabilization in levels of independence for performing daily activities, such as dressing, picking up objects and washing, as measured by the SMA Independence Scale (SMAIS-ULM).

Additionally, mean treatment adherence with Evrysdi over the multi-year period was over 99%. The overall rates of adverse events (AEs) and serious adverse events (SAEs) were reflective of the underlying disease and were consistent with previous data. No treatment-related AEs led to withdrawal from the study.

Muscle health and longer-term functional outcomes from individuals with Duchenne's disease after treatment with Elevidys, vs. well-matched external control groups

Elevidys showed statistically significant and clinically meaningful improvements across key measures of motor function two years after treatment in EMBARK (Phase III, NCT05096221) and three years after treatment in a pooled analysis of Study 101 (Phase I/II, NCT03375164, n=4), Study 102 (Phase II, NCT03769116, n=26), and ENDEAVOR Cohort 1 (Phase Ib, NCT04626674, n=20), compared to well-matched external control groups. Collectively, these data demonstrate that treatment with Elevidys results in long-term stabilisation or slowing of disease progression in individuals aged four through eight years of age at the time of treatment.

Topline data from year two of the EMBARK trial were announced in January 2025. No new safety signals were observed in the EMBARK study over the two-year duration.

To further evaluate the effect of Elevidys on disease progression, muscle health and changes in muscle pathology were assessed by magnetic resonance imaging (MRI) in a subset of individuals in EMBARK part one. At week 52, results showed stabilisation or slowing of disease progression in Elevidys-treated patients compared to placebo-treated patients. At week 104, MRI changes from baseline continued to generally favour Elevidys versus placebo at week 52 across muscles and muscle groups.

Further information on the abstracts that Roche will present at MDA 2025 can be found below.

Topic	Abstract Title	Presentation Number/Presentation Details
DMD	Long-term functional outcomes, safety, and micro-dystrophin expression following delandistrogene moxeparvovec treatment in DMD: EMBARK two-year results	#169 oral presentation
	Three-Year Functional Outcomes of Patients With Duchenne Muscular Dystrophy: Pooled Delandistrogene Moxeparvovec Clinical Trial Data vs External Controls	#167 oral presentation
	Assessment of Cardiac Outcomes in Delandistrogene Moxeparvovec Clinical Trials for Duchenne Muscular Dystrophy	#73 poster presentation
	Muscle MRI outcomes in patients with Duchenne Muscular Dystrophy treated with delandistrogene moxeparvovec: Findings from EMBARK Part 1	#168; poster presentation
	Long-Term Safety and Tolerability of Delandistrogene Moxeparvovec in Duchenne Muscular Dystrophy: Phase 1 to Phase 3 Clinical Trials	#89 poster presentation
	Impact of satralizumab on bone strength and muscle function in Duchenne muscular dystrophy (DMD): design of the SHIELD-DMD study	#82 poster presentation
	Assessing biomarkers of bone metabolism and the role of the interleukin (IL)-6 signalling pathway in	#146 poster presentation

	patients with Duchenne muscular dystrophy	
	Natural history of bone health in Duchenne muscular dystrophy: A systematic review and implications for the design of a clinical trial	#145 poster presentation
	Longitudinal Stride-Level Evaluation of Ambulatory Function with Ankle Wearable Technology in Ambulant DMD Patients Below 4 Years Old	#92 poster presentation
SMA	SUNFISH Parts 1 and 2: Five-year efficacy and safety data of risdiplam in Types 2 and 3 spinal muscular atrophy	#94 poster presentation
	Real-world risdiplam effectiveness in adults with spinal muscular atrophy (SMA) from the Pediatric Neuromuscular Clinical Research (PNCR) registry	#391 poster presentation
	RAINBOWFISH: Two-year efficacy and safety data in risdiplam-treated infants with presymptomatic spinal muscular atrophy (SMA)	#281 oral presentation Session: Clinical Trial Updates

About Duchenne muscular dystrophy

Duchenne is a rare, genetic, muscle-wasting disease that progresses rapidly from early childhood. Approximately one in 5,000 boys worldwide are born with Duchenne, while Duchenne in girls is very rare. Everyone who has Duchenne will lose the ability to walk, upper limb, lung and cardiac function and mean life expectancy is 28 years. A diagnosis of Duchenne will require full-time caregiving which is most often provided by parents, the majority of whom will find it difficult to carry out usual work or household activities and suffer from depression and physical pain.

Duchenne is caused by mutations of the *DMD gene*, which affects the production of the muscle protein, dystrophin. Dystrophin is a critical component of a protein complex that strengthens muscle fibers and protects them from injury during muscle contraction. Due to a genetic mutation in the *DMD gene*, people with Duchenne do not make functional dystrophin; their muscle cells are more sensitive to injury and muscle tissue is progressively replaced with scar tissue and fat. As dystrophin is also deficient in vital organ systems such as the cardiovascular and respiratory systems, the effect is thus inevitably fatal, with an average survival limited to the third decade of life.

About spinal muscular atrophy

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 or deletion of the survival motor neuron 1 (*SMN1*) gene, which leads to a deficiency of functional SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Currently approved drugs, including Evrysdi, prevent degeneration or death of these cells, 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 Evrysdi® (risdiplam)

Evrysdi is a survival motor neuron 2 (*SMN2*) pre-mRNA splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to survival motor neuron (SMN) protein deficiency. Evrysdi is administered daily at home in liquid form either by feeding tube or by mouth. In the United States a room-temperature stable Evrysdi 5 mg tablet formulation is also now available.

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 motor functions such as swallowing, speaking and breathing.

Evrysdi is currently approved in more than 100 countries, with more than 18,000 people with SMA treated globally.

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

About ELEVIDYS™ (delandistrogene moxeparvovec, SRP-9001)

Elevidys is the first approved disease-modifying gene therapy for Duchenne and is designed to address the underlying cause of Duchenne through targeted skeletal, respiratory and cardiac muscle expression of shortened dystrophin produced by Elevidys. Elevidys is a one-time treatment administered through a single intravenous dose. Elevidys is contraindicated in individuals with any deletion in exons 8 and/or 9 in the DMD gene.

Elevidys is approved for people living with Duchenne aged four years old and over regardless of their ambulatory status in the US, United Arab Emirates (UAE), Qatar, Kuwait, Bahrain and Oman. Elevidys is also approved for the treatment of ambulatory individuals aged four through seven years in Brazil and Israel. Filings have also been submitted to the European Medicines Agency (EMA) and regulatory authorities in Japan, Switzerland, Singapore, Hong Kong and Saudi Arabia. To date, more than 600 patients have been treated with Elevidys.

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 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.

F. Hoffmann-La Roche Ltd
4070 Basel
Switzerland
Group Communications
Roche Group Media Relations
Phone +41 61 688 88 88
www.roche.com

Roche Global Media Relations

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

Hans Trees, PhD
Phone: +41 79 407 72 58

Nathalie Altermatt
Phone: +41 79 771 05 25

Simon Goldsborough
Phone: +44 797 32 72 915

Nina Mählitz
Phone: +41 79 327 54 74

Yvette Petillon
Phone: +41 79 961 92 50

Sileia Urech
Phone: +41 79 935 81 48

Lorena Corfas
Phone: +41 79 568 24 95

Karsten Kleine
Phone: +41 79 461 86 83

Kirti Pandey
Phone: +49 172 6367262

Dr Rebekka Schnell
Phone: +41 79 205 27 03

Roche Investor Relations

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

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

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

Investor Relations North America

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