Media & Investor Release

Roche provides regulatory update on Elevidys™ gene therapy for Duchenne muscular dystrophy in the EU

- **EMA's CHMP issued an opinion not to recommend Elevidys™ (delandistrogene moxeparvovec) for the treatment of ambulatory individuals with Duchenne muscular dystrophy (DMD)**
- **Roche will continue its dialogue with the EMA to explore a potential path forward to make Elevidys available to individuals living with DMD in the EU**
- **Roche believes the benefit-risk remains positive in the ambulatory Duchenne population**
- **Elevidys is the first and only disease-modifying gene therapy for DMD**

Basel, 25 July 2025 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) issued a negative opinion on the conditional marketing authorisation (CMA) for Elevidys™ (delandistrogene moxeparvovec) for ambulatory individuals aged three to seven years with Duchenne muscular dystrophy (DMD). Given the high unmet need in DMD, Roche plans to continue to work with the EMA to explore a potential path forward.

"We are disappointed by the CHMP's negative opinion, given the urgent need for disease-modifying therapies for children in the EU living with Duchenne," said Levi Garraway, M.D., Ph.D., Chief Medical Officer and Head of Global Product Development, Roche. "With an average life expectancy of only 28 years, achieving disease stabilisation is a major advance for individuals living with Duchenne, their families and caregivers. We are confident in the value Elevidys can bring to ambulatory patients."

The CHMP opinion is based on data from the largest and broadest gene therapy clinical programme in DMD to date, including results from the pivotal Phase III EMBARK study that showed treatment with Elevidys provided sustained stabilisation or slowing of disease progression, and a consistent and manageable safety profile in ambulatory patients. To date, more than 900 individuals with DMD, 760 of whom are ambulatory, have been treated with Elevidys in clinical and real-world settings.

While the primary endpoint was not met in EMBARK after one year, Elevidys showed clinically meaningful and statistically significant improvements across important secondary endpoints of functional outcome measures when compared to placebo. Longer term efficacy data were also submitted to EMA, including two-year results from the EMBARK study and three-year pooled efficacy analysis from three other Elevidys studies that showed clinically meaningful improvements across key measures of motor function. One-year data from part one of the

EMBARK study were published in Nature Medicine in October 2024 and results from year two were shared at this year's Muscular Dystrophy Association clinical & scientific conference in Dallas, TX.

DMD is a rare, genetic, muscle-wasting disease that progresses rapidly from early childhood. Everyone with Duchenne will eventually lose the ability to walk, along with upper limb, lung, and cardiac function. Average life expectancy is only 28 years. The physical, emotional, and financial impact of Duchenne on those affected, their families, and caregivers, is profound. Roche recognises there is a significant unmet medical need for those living with Duchenne and the urgency for treating children before DMD progresses to provide the best possible chance for improved outcomes. We are actively working with health authorities across the globe to bring Elevidys to patients and their families as soon as possible.

Elevidys is the first and only approved gene therapy targeting the underlying cause of disease that consistently demonstrates stabilisation or slowing of DMD disease progression, with durable effects on functional and biological outcomes and muscle health.

About Duchenne muscular dystrophy

DMD primarily affects males, with 1 in 5,000 boys born worldwide having Duchenne. Everyone with Duchenne will eventually lose the ability to walk, along with upper limb, lung and cardiac function. Average life expectancy is only 28 years. The physical, emotional and financial impact of Duchenne on those affected, their families and caregivers, is profound.

Duchenne is an X-linked, rare neuromuscular disease caused by pathogenic variants (mutations) in the DMD gene that disrupt the production of functional dystrophin protein, leading to progressive and irreversible muscle weakness, diminished quality of life and premature death. Dystrophin strengthens and protects muscles and without it, normal activity causes excessive damage to muscle cells as they are more sensitive to injury. Over time, muscle tissue is replaced with scar tissue and fat, causing muscles to weaken. Although Duchenne progresses differently in each individual, its devastating trajectory is well established. Those with Duchenne will eventually lose the ability to use and move their limbs, to breathe on their own and are susceptible to respiratory infections. Muscle damage to the heart causes cardiomyopathy, including rhythm abnormalities and heart failure.

Early diagnosis is important for timely intervention to prolong muscle function and preserve quality of life. There is a critical need for disease-modifying treatments that address the underlying cause of DMD before irreversible muscle loss occurs.

About Elevidys™ (delandistrogene moxeparvovec)

Elevidys™ (delandistrogene moxeparvovec, also known as SRP-9001) 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 has been studied in the largest and broadest gene therapy clinical development program in DMD with the longest follow-up (up to six years) in patients of all ages, and with various DMD mutations. To date, more than 900 individuals with DMD (more than 760 of whom are ambulatory) have been treated with Elevidys across clinical and real-world settings and Elevidys is now approved in nine countries, including Japan.

Important Elevidys Updates:
On 15 June, Roche announced new dosing restrictions for Elevidys for non-ambulatory DMD patients, irrespective of age, in both clinical and commercial settings. These measures followed two reported fatalities in the non-ambulatory DMD population. On 22 July, in response to the U.S. Food and Drug Administration (FDA)'s request to Sarepta, Roche took additional measures towards initiating a voluntary and temporary pause of any new orders of Elevidys to countries outside the U.S. that reference the FDA as the basis for their local approval, and in Named Patient Supply (NPS) countries. Discussions with other relevant health authorities are ongoing. Roche will immediately respect requests to halt new orders and shipments from health authorities.

Patient safety is Roche's highest priority. Based on the totality of available data, Roche believes that the benefit-risk profile is positive in the ambulatory patient population. To date, approximately 760 ambulatory DMD patients have been treated with Elevidys in clinical and real-world settings and there have been no treatment-related fatalities.

Elevidys is being developed by Roche in collaboration with Sarepta Therapeutics

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.

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