Investor Update

Roche announces EMA has initiated review of the Elevidys Marketing Authorisation application for the treatment of Duchenne muscular dystrophy (DMD)

- **If approved, Elevidys is expected to be the first and only gene therapy to address the underlying cause of Duchenne, available in Europe**
- **Elevidys is already approved in the US, Qatar, Kuwait, UAE, Oman and Bahrain**

Basel, 24 June 2024 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the European Medicines Agency (EMA) has validated and initiated review of the marketing authorisation application (MAA) for Elevidys™ (delandistrogene moxeparvovec), a gene therapy for the treatment of ambulatory patients aged 3-7 years old with Duchenne muscular dystrophy.

"Duchenne is a devastating muscle-wasting disease with no cure. Treatments that can change the disease course and preserve muscle function are urgently needed," said Levi Garraway, M.D., Ph.D., Roche's Chief Medical Officer and Head of Global Product Development. "Roche is committed to bringing Elevidys to the children who need it and we welcome the EMA's review of the filing submission."

The Elevidys MAA has been submitted to the EMA and is based on results from the pivotal Phase 3 EMBARK study, a global, randomised, double-blind, placebo-controlled study in patients with Duchenne aged 4 through 7 years. Although the EMBARK study did not meet the primary endpoint, the totality of evidence from the trial confirms that Elevidys is the first gene therapy to provide clinically meaningful benefits for patients by modifying the disease course, with a manageable safety profile. In EMBARK, Elevidys-treated patients had clinically meaningful and statistically significant benefits in both key secondary functional endpoints: time to rise from floor and 10 minute walk/run test. Additionally, a clinically meaningful and statistically significant improvement was also observed for the pre-specified secondary endpoint: stride velocity 95th centile. This novel digital endpoint, qualified by the EMA, measures speed of walking via a wearable device (Syde®). The time to ascend 4-steps secondary endpoint also demonstrated consistent treatment benefit in favour of Elevidys. The MAA is also supported by data from ENDEAVOR, an open- label study in patients with Duchenne, that is enrolling ambulatory and non-ambulatory patients of various ages, along with a Phase I/II study

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which provides longer term efficacy, durability, safety and biological data to support the benefit-risk assessment.

In addition, results from two other studies are being conducted to expand the label: ENVOL, a Phase 2 study in boys with Duchenne under the age of 4 and ENVISION, a Phase 3 study in older ambulatory and non-ambulatory patients with DMD.

As part of a 2019 collaboration agreement with Sarepta, Roche is responsible for bringing Elevidys to patients outside the US. Approval has already been granted in five countries that can accept applications for marketing authorisation based on Phase 1 and Phase 2 data, making it the first and only gene therapy for Duchenne approved in these countries. Elevidys is also under review in four other countries and further regulatory submissions of Elevidys are underway.

Following the acceptance of an efficacy supplement by the FDA in December 2023, Sarepta recently announced that the FDA has converted the accelerated approval to a traditional approval and expanded the label to include ambulatory individuals with a confirmed mutation in the DMD gene who are aged four and older. In addition, the FDA also granted accelerated approval to Elevidys for non-ambulatory Duchenne patients.

About Elevidys™

Elevidys™ (delandistrogene moxeparvovec, also known as SRP-9001) is the first approved disease-modifying 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 (one-time) intravenous dose. Elevidys is contraindicated in patients with any deletion in exons 8 and/or 9 in the *DMD gene*.

Elevidys received accelerated approval in the US in June 2023, and is now approved in the United Arab Emirates, Qatar, Kuwait, Bahrain and Oman for the treatment of ambulant children aged 4 through 5 years with Duchenne, who have a confirmed mutation in the *DMD gene*.

About Duchenne muscular dystrophy

Duchenne is a rare, genetic, muscle-wasting disease that progresses rapidly from early childhood. Approximately 1 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 DMD 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, pain and discomfort.

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 fibres 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 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 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 recognising our endeavour 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 fifteenth 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.

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