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PRESS RELEASE

Novartis provides update on delpacibart etedesiran (del-desiran) Phase III HARBOR study for the treatment of myotonic dystrophy type 1 (DM1)

Ad hoc announcement pursuant to Art. 53 LR

- *HARBOR did not meet its primary endpoint of vHOT in DM1, a progressive neuromuscular disease with high unmet need and no approved treatment options*
- *Del-desiran showed evidence of clinical activity in secondary endpoints and exploratory analyses; full HARBOR dataset to be analyzed and appropriate development path to be determined*
- *AOC pipeline continues to advance with FDA priority review granted for delpacibart zotadirsen in DMD44 and planned FDA meeting for delpacibart braxlosiran in FSHD, based on positive Phase I/II biomarker data*
- *Novartis maintains its 5-6% five-year sales CAGR guidance for 2025-2030*

Basel, September 8, 2026 – Novartis today announced that the global Phase III HARBOR study evaluating del-desiran in people living with myotonic dystrophy type 1 (DM1) did not demonstrate statistically significant improvement versus placebo on the primary endpoint of video hand opening time (vHOT), a novel measure of hand myotonia.[1,2] Evidence of clinical activity in secondary endpoints and exploratory analyses were observed. Safety findings from HARBOR were generally consistent with previously reported data. Novartis is evaluating the full HARBOR dataset and will engage with health authorities to determine the most appropriate development path for del-desiran.

“Despite decades of research, there are still no approved treatment options for DM1, and patients and caregivers continue to face a significant daily burden,” said Shreeram Aradhye, President, Development and Chief Medical Officer, Novartis. “Developing therapies for a complex disease like DM1 remains challenging, and setbacks are part of scientific progress. As we continue to evaluate the full HARBOR dataset, we remain committed to identifying the most appropriate development path for the del-desiran program and advancing innovative approaches for people living with DM1 and other serious neuromuscular diseases.”

Del-desiran is one of three antibody oligonucleotide conjugate (AOC) therapies added to the Novartis neuromuscular pipeline through the acquisition of Avidity Biosciences. Novartis is advancing delpacibart zotadirsen (del-zota) in patients with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44). The company filed del-zota for accelerated approval and was granted priority review designation by US Food and Drug Administration (FDA). Novartis is planning to meet with the FDA on next steps for delpacibart braxlosiran (del-brax) in facioscapulohumeral muscular dystrophy (FSHD) based on recent positive Phase I/II biomarker data.

About DM1
Myotonic dystrophy type 1 (DM1) is a progressive, multisystem, heterogeneous neuromuscular disease caused by an expansion of CTG repeats in the DM1 protein kinase (*DMPK*) gene.[3,4,5] People living with DM1 may experience a range of internal and systemic symptoms, including myotonia, muscle weakness and impaired hand function,

which can affect everyday activities, independence, and quality of life.[3]

About Del-desiran
Del-desiran is an investigational antibody oligonucleotide conjugate (AOC) designed to target the underlying cause of DM1. The therapy consists of a muscle-targeting monoclonal antibody that binds to the transferrin receptor 1 (TfR1) and is conjugated to a small interfering RNA (siRNA) designed to induce degradation of disease-causing toxic DMPK messenger RNA (mRNA).[6] Del-desiran received Orphan Drug, Fast Track and Breakthrough Therapy Designations from the US Food and Drug Administration (FDA), and Orphan Medicinal Product Designation in the European Union (EU).

About the HARBOR study
HARBOR (NCT06411288) is a global Phase III, randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of del-desiran over 54 weeks in approximately 150 people living with DM1. Participants were randomized to receive del-desiran or placebo every eight weeks. The study assesses the impact of del-desiran across multiple functional aspects of DM1. The primary endpoint is vHOT and key secondary endpoints include muscle strength as measured by hand grip strength and quantitative muscle testing (QMT) total score, activities of daily living as measured by DM1-Activ, and mobility and physical function as measured by the 10-meter walk/run test (10mWRT).[1]

Novartis in Neuroscience
Neurological diseases are deeply personal, affecting people of any age, from newborns to seniors, often striking in the prime of life. At Novartis, we are doubling down on our commitment to neurology, expanding our legacy of innovation in spinal muscular atrophy (SMA) and multiple sclerosis (MS) to work in neuroimmunology, neurodegeneration, and neuromuscular diseases. Our goal is to protect people's health across their lifespan, developing more treatment options that lead to better outcomes.

Disclaimer
This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as "potential," "can," "will," "plan," "may," "could," "would," "expect," "anticipate," "progressive," "continues," or similar expressions, or by express or implied discussions regarding: potential new products including del-desiran, del-zota or del-brax; potential new indications for existing products; potential product launches or potential future revenues from del-desiran, del-zota or del-brax; results of ongoing clinical trials; or potential future, pending or announced transactions; or potential future sales or earnings from del-desiran, del-zota or del-brax. You should not place undue reliance on these statements. Such forward-looking statements are based on the current beliefs and expectations of management regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that del-desiran, del-zota or del-brax will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that del-desiran, del-zota or del-brax will be commercially successful in the future. In particular, our expectations could be affected by, among other things, uncertainties concerning: global healthcare cost containment, including ongoing government, payer and general public pricing and reimbursement pressures and requirements for increased pricing transparency; the success of our key products, commercial priorities and strategy; research and development of new products, including clinical trial results and additional analysis of existing clinical data; our ability to obtain or maintain proprietary intellectual property protection, including the ultimate extent of the impact on Novartis of the loss of patent protection and exclusivity on key products; our ability to realize the strategic benefits, operational efficiencies or opportunities expected from our external business opportunities; the development or adoption of new technologies, including artificial intelligence, and new business models; potential significant breaches of information security or disruptions of our information technology systems; actual or potential legal proceedings, including regulatory actions or delays or government regulation related to the products and pipeline products described in this press release; safety, quality, data integrity, or manufacturing issues; major macroeconomic and geo- and socio-political developments, including the impact of any potential tariffs on our products or the impact of war in certain parts of the world; future global exchange rates; future demand for our products; and other risks and factors referred to in Novartis AG's most recently filed Form 20-F and in subsequent reports filed with, or furnished to, the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-

looking statements as a result of new information, future events or otherwise.

About Novartis
Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach more than 300 million people worldwide.

Reimagine medicine with us: Visit us at **www.novartis.com** and connect with us on **LinkedIn**, **Facebook**, **X/Twitter** and **Instagram**.

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