NOVARTIS

Community Update: Delpacibart etedesiran (del-desiran) Phase III HARBOR study for the treatment of myotonic dystrophy type 1 (DM1)

September 8, 2026

Dear Myotonic Dystrophy Community,

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. Evidence of clinical activity in secondary endpoints and exploratory analyses were observed. Safety findings from HARBOR were generally consistent with previously reported data.

We know how much the entire DM1 community contributes to research efforts like HARBOR, and we recognize these results are not what everyone hoped for. As we continue to evaluate the full HARBOR dataset, we will engage with health authorities to determine the most appropriate development path for del-desiran. Novartis remains committed to advancing innovative approaches for people living with DM1 and other serious neuromuscular diseases including Duchenne muscular dystrophy (DMD), spinal muscular atrophy (SMA) and facioscapulohumeral muscular dystrophy (FSHD).

We are deeply grateful to the people with DM1 who participated in the HARBOR study and their families, as well as the investigators, clinical sites worldwide, researchers, advocates, and DM1 community organizations whose support made this research possible. These contributions have been essential to deepening the understanding of and advancing research and innovation for this disease, not only for our organization but also for the broader community.

With gratitude,
The del-desiran Clinical Development Team at Novartis
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