

An Open Letter to Novartis from the Global Myotonic Dystrophy Community

September 15, 2026

To the leadership of Novartis,

Today, on [International Myotonic Dystrophy Awareness Day](#), we write as patient advocacy organizations, including families, clinicians, researchers, and people living with myotonic dystrophy around the world.

We write with profound disappointment following the [announcement that the Phase 3 HARBOR](#) study of del-desiran did not meet its primary endpoint, video Hand Opening Time (vHOT).

We recognize that clinical development is difficult, that scientific setbacks happen, and that a Phase 3 trial failing to meet its primary endpoint is devastating for everyone involved. We also recognize the enormous effort, expertise, resources, and commitment that Avidity/Novartis and the HARBOR investigators have invested in this program.

But we ask you, respectfully and urgently:

Please do not abandon the myotonic dystrophy program.

For people living with myotonic dystrophy, this is not simply a clinical trial result. **It is our lives.**

It is the child growing up wondering why their body will not work as it should. It is the young adult wondering how long they will be able to work, walk, drive, or care for their family. It is the parent watching a son or daughter lose abilities most people take for granted. It is the person struggling with grip and hand function, mobility, fatigue, swallowing, breathing, cardiac complications, pain, and the many other manifestations of this devastating, multisystem disease.

And it is the families who have spent years waiting for something that could fundamentally change the course of this disease - and their lives.

The myotonic dystrophy community has lived without an approved disease-modifying treatment since the disease was first identified in 1909. Novartis itself has acknowledged that DM1 remains a disease with high unmet need and no approved treatment options.

That is why the HARBOR announcement is so painful.

But, there is another part of your announcement that gives this community reason to hope. You stated that del-desiran showed evidence of clinical activity in secondary endpoints and exploratory analyses. You also stated that the full HARBOR dataset is being analyzed and

that Novartis will engage with health authorities to determine the most appropriate development path.

We ask you to follow that evidence.

The primary endpoint is not the entire story. We understand its importance and that regulatory decisions must be based on rigorous scientific evidence. But myotonic dystrophy is an extraordinarily complex, multisystem disease. A single measure of hand opening time cannot capture the totality of what matters to people living with DM1.

Therefore, as a united global community, we ask Novartis to fully evaluate, disclose, and pursue the clinical meaning of the secondary and exploratory findings from HARBOR.

Please:

- Complete a comprehensive analysis of all secondary and exploratory endpoints, including measures of muscle function, mobility, myotonia, patient-reported outcomes, quality of life, disease biology, and other clinically meaningful outcomes.
- Examine whether particular subsets of HARBOR participants, disease stages, treatment durations, or other characteristics responded differently.
- Make the results available to the medical and scientific community as rapidly and transparently as possible.
- Engage global regulatory authorities and independent experts regarding potential paths forward.
- Investigate whether participant subsets or clinically meaningful effects warrant further study.
- Consider whether additional follow-up, alternative endpoints, better patient characterization, trial-design changes, or other approaches could provide a clearer understanding of del-desiran's potential.
- Continue meaningful dialogue with people living with myotonic dystrophy and the organizations representing them before making a final decision about the program.

If the data truly identify a signal worth pursuing, please pursue it.

A failed primary endpoint should prompt careful scientific investigation—not necessarily the end of a program when other evidence may point toward biological or clinical activity.

Finish the analysis before making a final decision.

Novartis has said that the full HARBOR dataset is still being analyzed. We therefore ask you **not to make an irreversible decision about the future of del-desiran before that analysis is complete.**

We are not asking Novartis to ignore the HARBOR result. **We are asking you to understand it completely.**

Please remember the people behind the data.

Hundreds of people with DM1 volunteered to participate in HARBOR, knowing the uncertainties and risks of clinical research. They traveled to trial sites, often hundreds or thousands of miles away every few weeks. They underwent assessments, received del-desiran or placebo, and gave their time, data, bodies, and hope to this research.

Their participation created knowledge that did not exist before.

The least we can ask is that every meaningful piece of information generated from that sacrifice be pursued to its fullest scientific potential.

Those participants did not fail. The community did not fail. Science encountered an unexpected result.

Now we ask Novartis to respond with the same courage that patients demonstrated when they entered the trial.

This is bigger than one drug.

We understand that Novartis must make difficult decisions about its pipeline and investment priorities. But the importance of the del-desiran program extends beyond a single molecule.

The HARBOR trial represents one of the largest and most significant efforts ever undertaken to develop a disease-modifying therapy for myotonic dystrophy. Walking away now could have consequences far beyond one program: it could discourage investment in myotonic dystrophy, undermine confidence in the field, and leave a devastating disease without a clear path toward treatment.

Conversely, carefully investigating the complete HARBOR dataset could advance scientific understanding even if del-desiran ultimately does not become an approved therapy.

There is still knowledge to be gained. There are still questions to answer. And there may still be a path forward.

To the people at Avidity and Novartis who have worked on this program:

We want you to know that we see you, too.

We know that scientists, physicians, clinical investigators, study coordinators, advocates, and countless others have dedicated years of their lives to this effort. We know you wanted this trial to succeed, and we know this result is disappointing for you as well.

But precisely because so much work has gone into HARBOR, we ask you not to let the story end with the primary endpoint.

Your own announcement tells us that there are secondary and exploratory findings that deserve further examination. **Please follow those findings carefully, as if lives depend on it—because they do.**

This is a plea from the global myotonic dystrophy community.

We are asking Novartis to choose **persistence over abandonment.**

We are asking you to choose **scientific curiosity over premature closure.**

We are asking you to choose **partnership with patients.**

And above all, we are asking you to remember why this work matters.

Every day, people living with myotonic dystrophy wake up in bodies that are gradually losing function. Every day, families adapt their lives around a disease they cannot stop. Every day, people wait for researchers to find an answer.

We cannot promise you that del-desiran is the answer.

But we can tell you that the question is far too important to stop asking when your own data indicate that there may still be something there to investigate.

Please complete the analysis.

Please explore the secondary endpoints.

Please explore the exploratory endpoints.

Please engage with regulators and independent experts.

Please talk with the global patient community.

And please—do not abandon myotonic dystrophy.

For the people who participated in HARBOR, for the families who supported them, for the researchers who have dedicated their careers to this disease, for the generations who will inherit myotonic dystrophy, and for the hundreds of thousands—possibly millions—of people and families around the world whose lives are affected by, or will be affected by, myotonic dystrophy and who are still waiting for hope to become a treatment:

We ask Novartis to stay.

We ask you to finish the work.

We ask you to follow the science wherever it leads.

With respect, hope, and an unwavering commitment to the myotonic dystrophy community,

The Global Myotonic Dystrophy Patient Advocacy Community



**Muscular Dystrophy
Australia (Australia)**



**Muscular Dystrophy
Canada (Canada)**



Euro-DyMA (Europe)
Representing 14 countries.



MD-Europe (Europe)



**Myotonic Dystrophy
Patients' Group of Japan
(DM-Family) (Japan)**



**MDT1F (Myotonic
Dystrophy Type 1 Family) –
Korean Myotonic
Dystrophy Patient Group
(Korea)**



**Spierziekten Nederland
(Netherlands)**



ADM1ES (Spain)



**Myotonic Dystrophy
Support Group
(United Kingdom)**



**Myotonic Dystrophy
Foundation (MDF) (USA)**