26 June 2024

Dear Members of the DM1 Community,

We are very excited to share that we have initiated and are enrolling patients in the global Phase 3 HARBOR™ clinical trial for the treatment of adults living with myotonic dystrophy type 1 (DM1). This is a monumental milestone for the DM1 community and for Avidity as we work together to develop a treatment to address the underlying cause of DM1.

The global HARBOR Phase 3 study is investigating delpacibart etedesiran (formerly AOC 1001; abbreviated as del-desiran) for people aged 16 and over who have a clinical and genetic diagnosis of DM1. To learn more about the study including an up-to-date list of clinical trial sites currently recruiting, please go to the following link: clinicaltrials.gov.

At this time, there are two centers currently active and recruiting patients. Our plan is to open approximately 40 global sites. We are preparing multiple other sites in the US, Canada and select countries across Europe and Asia Pacific. Over the next several weeks and months, more sites will begin recruiting. Please know that while initiating a global clinical trial takes time, we are committed to getting the different countries and sites open as quickly as we can. As additional clinical trial sites initiate, we will continue to update the HARBOR Phase 3 clinical trial listing on the clinicaltrials.gov website and keep the Myotonic Dystrophy Foundation (MDF) apprised of our progress.

We are incredibly encouraged by the growing body of data supporting the safety and efficacy of del-desiran, including the recent positive long-term data from the ongoing MARINA open-label extension (MARINA-OLE™) trial. The favorable long-term safety data and consistent, durable improvement in myotonia, muscle strength and patient-reported outcome measures show the potential of del-desiran to make a meaningful difference in the lives of people living with DM1.

We are working to finalize this study as quickly as we can and gather the data necessary for approval. We have heard from many of you and understand how important it is to make del-desiran available for those who need it.

We want to thank the entire patient community for your time, commitment and continued contributions to the development of del-desiran. Together we have accomplished so much, and we look forward to a continued partnership with the community as we move into this next phase of development. We are so grateful to the current and future participants, their families, the investigators and their teams as we work together to advance del-desiran in clinical development. We remain steadfast in our commitment to advance meaningful therapies for the muscular dystrophy community.

We encourage you to contact your doctor if you have any questions about del-desiran or the HARBOR trial.

Sincerely,
The Avidity Team