

July 28, 2025

Dear DM1 Community,

Today, we are very pleased to share with you that we have completed enrollment in the ongoing global Phase 3 HARBOR™ clinical trial of delpacibart etedesiran (del-desiran) in people living with myotonic dystrophy type 1 (DM1). This marks a significant step for both the DM1 community and Avidity as we work together to advance a treatment that addresses the underlying genetic cause of DM1.

The HARBOR trial is designed to assess the impact of del-desiran 4 mg/kg administered every eight weeks versus placebo for 54 weeks in people living with DM1, aged 16 and over. Avidity anticipates topline data from HARBOR in the second quarter of 2026. We are also on track to share updates from the ongoing MARINA open-label extension (MARINA-OLE™) trial of del-desiran including long-term 4 mg/kg efficacy and safety data in the fourth quarter of 2025.

We plan to submit marketing applications for del-desiran beginning in the second half of 2026 including in the U.S., European Union and Japan, depending on the results of the HARBOR clinical study.

You can view our full press release of today's DM1 news here:

https://investors.aviditybiosciences.com/2025-07-28-Avidity-Biosciences-Announces-Completion-of-Enrollment-for-HARBOR-TM-,-the-First-Global-Phase-3-Trial-of-Delpacibart-Etedesiran-del-desiran-for-Treatment-of-DM1-and-Provides-Guidance-on-Regulatory-Submission

We are working to finalize the HARBOR study as quickly as we can and gather the data necessary for regulatory submissions and, ultimately, approval. Completing enrollment in the HARBOR study for del-desiran is a significant step forward in bringing potentially the first approved drug for DM1.

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 study 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 and MARINA-OLE trials.

Sincerely,

The Avidity Team