



May 8, 2024

We are pleased to share that the Food and Drug Administration (FDA) granted delpacibart etedesiran (formerly AOC 1001; abbreviated as del-desiran) Breakthrough Therapy designation.

Breakthrough Therapy designation is granted by FDA to expedite the development and review of drugs for serious or life-threatening conditions. In order to achieve breakthrough therapy designation, there must be preliminary clinical evidence that demonstrates the drug may have a substantial improvement on at least one clinically significant endpoint over currently available therapies.

This designation from FDA highlights the potential of del-desiran to be an effective treatment and underscores the urgency of bringing this potential treatment to people living with DM1. You can view our full press release of today's DM1 news here: [Avidity Biosciences Receives FDA Breakthrough Therapy Designation for Delpacibart Etedesiran \(AOC 1001\) for Treatment of Myotonic Dystrophy Type 1](#)

We want to thank the entire patient community for your time, commitment and continued contributions to the development of del-desiran. In particular, we are so grateful to the participants in the MARINA[®] and MARINA-OLE[™] studies, their families, the investigators and their teams. Your participation in MARINA generated the data that supports this designation as well as the data to advance del-desiran to the next stage of development.

We are also very grateful to the clinicians, researchers and participants in the END-DM1 natural history study and the Myotonic Dystrophy Clinical Research Network (DMCRN). Data from END-DM1 has provided important information about the natural progression of DM1 that further demonstrates the need for a treatment.

Looking ahead, we are in the process of initiating the HARBOR[™] global phase 3 study and will share information with the community as it becomes available. We share the urgency for a treatment for people living with DM1 and we remain hopeful about the potential of del-desiran (AOC 1001). We remain steadfast in our commitment to advance meaningful therapies for the myotonic dystrophy community.

We encourage you to contact your doctor if you have any questions about del-desiran (AOC 1001).

Sincerely,

The Avidity Team