



April 27, 2023

We are pleased to inform you that today we announced positive topline data from the Phase 1/2 MARINA™ study of AOC 1001 in adults living with myotonic dystrophy type 1 (DM1).

Topline data from the MARINA trial show that AOC 1001 demonstrated functional improvement, disease modification and has a favorable safety and tolerability profile. The AOC 1001 topline data were highlighted in an oral presentation at the 75th American Academy of Neurology (AAN) Annual Meeting in Boston, Mass. You can view our full press release on our corporate website (<https://aviditybiosciences.investorroom.com/news-releases>).

We are also planning to review the topline data in a live webinar in partnership with the Myotonic Dystrophy Foundation (MDF) on Tuesday, May 9th at 1:00pm PT (4:00pm ET). We invite the DM1 community to register to join the webinar on MDF's website (<https://www.myotonic.org/meet-dm-drug-developers-avidity-biosciences-2>). We hope that you will be able to join us for this planned presentation.

In people living with DM1, AOC 1001 Phase 1/2 topline data demonstrated:

- Directional improvement in multiple functional assessments including measures of myotonia, strength and mobility:
- Meaningful DMPK reduction and splicing changes in participants treated with AOC 1001
 - Splicing changes followed by directional improvements in functional measures at 2 mg/kg and 4mg/kg doses of AOC 1001
 - AOC 1001 demonstrated broad splicing improvements in more than a thousand genes impacted by DM1, confirming activity in the nucleus
- Favorable safety and tolerability profile of AOC 1001 with most adverse events mild or moderate

The topline data from the MARINA trial reinforce our belief in the potential for AOC 1001 to be an effective treatment for people living with the devastating impact of DM1. Data from MARINA delivered a robust data package that we believe supports discussions with regulatory agencies about the design of a pivotal study. We are committed to designing the quickest path to bring AOC 1001 to patients.

We continue to dose 36 participants at both 2 mg/kg and 4 mg/kg of AOC 1001 in the MARINA open-label extension (MARINA-OLE™) study to evaluate the long-term safety and tolerability of AOC 1001 in participants with DM1 who were previously enrolled in the Phase 1/2 MARINA trial. Avidity remains on track to share a first look at the data from the MARINA-OLE study at the end of 2023.

We remain steadfast in our commitment to advance meaningful therapies for the myotonic dystrophy community, and we want to thank each participant in the study, their families, our



advocacy partners as well as the investigators and their teams for their time, commitment, and continued contributions. We share the urgency for a treatment for people living with DM1 and we remain hopeful about the potential of AOC 1001.

We encourage you to contact your doctor if you have any questions about AOC 1001, the MARINA trial, or the MARINA-OLE trial.

Sincerely,

The Avidity Team