

October 7, 2023

We are pleased to inform you that today we announced new positive AOC 1001 data from the Phase 1/2 MARINA® clinical trial and MARINA open-label extension (MARINA-OLE™) trial in adults living with myotonic dystrophy type 1 (DM1).

The new AOC 1001 data demonstrated improvement in multiple additional functional measures including hand grip, muscle strength and patient reported outcomes in people living with DM1. New long-term safety data of AOC 1001 also continue to demonstrate favorable safety and tolerability. These data augment previously reported positive data showing improvements in myotonia, muscle strength and mobility reported at the American Academy of Neurology (AAN) Annual Meeting earlier this year in April.

The new positive AOC 1001 data were highlighted in an oral presentation at the 28th Annual Congress of the World Muscle Society (WMS) in Charleston. You can view our full press release on our corporate website (https://aviditybiosciences.investorroom.com/news-releases).

New positive AOC 1001 data presented at WMS include:

- Multiple additional measures of strength:
 - Hand grip
 - Manual Muscle Testing (MMT) composite score
 - Both upper and lower QMT composites
- DM1-Activ, a patient reported outcome (PRO) that measures activities of daily living (e.g., taking a shower, visiting family or friends, and walking up stairs).

Cumulatively between MARINA and MARINA-OLE, over 200 infusions have been administered, totaling 46.2 patient-years of exposure.

New favorable long-term AOC 1001 safety and tolerability data show that AOC 1001 was generally well tolerated with the most common adverse events (AEs) in the MARINA-OLE being procedural pain (22%), pain in extremity (such as arm, leg or foot pain/soreness) and headache (both 16%). All participants that enrolled in the MARINA-OLE remain in the study.

Data from MARINA and MARINA-OLE reinforce our belief that AOC 1001 has the potential to become an effective treatment option for people living with DM1. We are finalizing a Phase 3 study design and a global regulatory path for AOC 1001 and look forward to sharing a first look at efficacy data from the MARINA-OLE study in the first half of 2024.

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