

August 9, 2024

We are very pleased to share with you that today we announced positive initial data from our EXPLORE44™ clinical trial, that is assessing the safety and efficacy of our investigational therapy, AOC 1044 in people living with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44). EXPLORE44 is a placebo-controlled study that enrolled 40 healthy volunteers and 24 participants with DMD44, ages seven to 27 years old. AOC 1044 is designed to specifically skip exon 44 of the dystrophin gene to enable dystrophin production in people living with DMD44.

Data from the 5mg/kg cohort of AOC 1044 at four months demonstrated statistically significant increase of 25% of normal in dystrophin production, statistically significant increase of 37% in exon 44 skipping and reduced creatine kinase levels to near normal with greater than 80% reduction compared to baseline. AOC 1044 demonstrated favorable safety and tolerability in people living with DMD44.

We also shared today that *delpacibart zotadirsen* is the approved international nonproprietary name of AOC 1044, abbreviated as *del-zota*.

You can view our full press release announcing today's DMD44 news here:

[Avidity Biosciences Announces Positive AOC 1044 Data Demonstrated Significant Increase of 25% in Dystrophin Production and Reduction of Creatine Kinase Levels to Near Normal in People Living with Duchenne Muscular Dystrophy Amenable to Exon 44 Skipping in the Phase 1/2 EXPLORE44™ Trial](#)

In EXPLORE44 study, *del-zota* demonstrated:

- Consistent delivery of PMO of 200 nM in skeletal muscle
- Statistically significant 37% increase in exon 44 skipping and up to 66% skipping
- Statistically significant increase of 25% of normal in dystrophin production and restored total dystrophin up to 54% of normal
- Creatine kinase levels were reduced to near normal with greater than 80% reduction compared to baseline
- Favorable safety and tolerability with most treatment emergent adverse events (AEs) mild or moderate in participants with DMD44.

We would like to thank each participant in the trial, their families, our advocacy partners and the investigators and their teams for their time, commitment, and continued contributions.

To learn more about these findings, we invite you to join us for an online webinar in partnership with Cure Duchenne. The Avidity team, joined by Dr. Diana Castro, Pediatric Neurologist, Neurology and Neuromuscular Care Center and Founder and Director, Neurology and Neuromuscular Care Center/Neurology Rare Disease Center, will review the EXPLORE44 trial initial findings and provide insights on what these results mean for this development program and for the DMD44 community.

**Date:** Friday, August 16th, 2024

**Time:** 9:00am Pacific Time / 12:00pm Eastern Time

**How to join:** You can register to join the webinar on the Cure Duchenne website ([https://us02web.zoom.us/webinar/register/WN\\_fxeRaHKzRdGUMB7YMRrPhg](https://us02web.zoom.us/webinar/register/WN_fxeRaHKzRdGUMB7YMRrPhg))

A recording of this webinar will be available at a later date.

With this encouraging data, we will focus on regulatory discussions with FDA as we work to complete both our EXPLORE44 and EXPLORE44-OLE (Open-Label Extension) studies. While the EXPLORE44 trial is fully enrolled, it is important that participants complete participation. Our plan is to enroll an additional 10-15 patients in the EXPLORE44-OLE study.

We share the sense of urgency and the desire to bring new therapies to the DMD community as quickly as possible. Our most important mission is to complete both of these studies and seek out a pathway toward approval. We anticipate we will have requests for access including a Managed Access Pathway (known as Expanded Access or Compassionate Use in the US) which provides access to investigational therapies outside of a formal clinical trial setting. We will be discussing a potential MAP after we conclude our initial regulatory conversations. Del-zota is currently not available outside of the EXPLORE44 and EXPLORE44 OLE studies. If you have questions, please contact [patients@aviditybio.com](mailto:patients@aviditybio.com).

We encourage you to contact your doctor if you have any questions about *del-zota* or the EXPLORE44 trial.

Sincerely,

The Avidity Team