March 17, 2025

Dear Advocacy Partner,

Today, we are pleased to share with you positive topline data from the EXPLORE44® clinical trial, which assessed the safety and efficacy of our investigational therapy delpacibart zotadirsen (formerly AOC 1044, abbreviated as del-zota) in people living with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44).

The new positive del-zota topline data are being presented this week at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference. These data are consistent with the positive initial del-zota data we shared from this study in August 2024, including statistically significant improvements in dystrophin production, exon skipping and creatine kinase levels as well as favorable safety and tolerability across the dose cohorts.

We would like to extend our heartfelt gratitude to all participants in the EXPLORE44® clinical trial, as well as their families, and our advocacy partners and the investigators and their teams for their time and continued commitment.

You can view our full press release announcing today's news here: Avidity Biosciences Announces Positive Topline Del-zota Data Demonstrating Consistent, Statistically Significant Improvements in Dystrophin, Exon Skipping and Creatine Kinase in People Living with Duchenne Muscular Dystrophy Amenable to Exon 44 Skipping in Phase 1/2 EXPLORE44® Trial. We would also like to invite you to join us for a webinar in partnership with Parent Project Muscular Dystrophy (PPMD) on March 28 to discuss these data. Additional information on the webinar is below.

The data presented at MDA will highlight the consistent data across all parameters in both the 5mg/kg and 10 mg/kg cohorts of del-zota, including:

- Targeted delivery of PMOs resulting in tissue concentrations of approximately 200nM in skeletal muscle;
- Statistically significant increases of approximately 40% in exon 44 skipping;
- Statistically significant increase of approximately 25% of normal in dystrophin production and restored total dystrophin up to 58% of normal;
- Reduction in creatine kinase levels to near normal with greater than 80% reductions compared to baseline:
    - Similarly, placebo participants demonstrated a reduction in creatine kinase levels to near normal upon treatment with del-zota;
    - Significant reductions in creatine kinase levels were sustained in the EXPLORE44-OLE trial with continued treatment up to one year; and,
- Del-zota demonstrated favorable safety and tolerability at both doses, with most treatment emergent adverse events (TEAEs) mild or moderate.

Based on the consistent data between the 5 mg/kg every six weeks and the 10mg/kg every eight weeks groups across all parameters, Avidity has selected the dose of 5 mg/kg every six weeks of del-zota for the Biologics License Application (BLA) submission and future clinical studies.

Participants currently receiving the 10 mg/kg dose in the EXPLORE44-OLE trial are in the process of being transitioned to 5 mg/kg every six weeks.

Following alignment with FDA on an accelerated approval path late last year, including dose selection, we remain on track for a year-end BLA submission for del-zota. We also look forward to presenting topline results including functional data from the ongoing EXPLORE44-OLE study in the fourth quarter of this year. We continue to share the sense of urgency and the desire to bring new therapies to the DMD community as quickly as possible.

Upcoming Webinar Hosted by Parent Project Muscular Dystrophy (PPMD)

To learn more about these findings, we invite you to join us for a webinar in partnership with Parent Project Muscular Dystrophy (PPMD). The Avidity team, joined by Dr. Aravindhan Veerapandiyan (Dr. Panda), Associate Professor of Pediatrics, University of Arkansas for Medical Sciences and Arkansas Children's Hospital, will present the EXPLORE44 trial topline data, focusing on what these results mean for the DMD44 community.

Date: Friday, March 28, 2025

Time: 11:00am Pacific Time / 2:00pm Eastern Time

> **How to join:** You can register to join the webinar on PPMD's website via this link: Webinar Registration - Zoom: A recording will be available on PPMD's website within a week following the conclusion of the webinar.

We would like to again thank each participant in the trial, their families, our advocacy partners as well as the investigators and their teams for their time, commitment, and continued contributions. We remain steadfast in our commitment to advance meaningful therapies and are hopeful about the potential of del-zota to change the course of disease for people living with DMD44.

We encourage individuals, families and care partners to contact your doctor if you have any questions about del-zota, or the EXPLORE44 and EXPLORE44-OLE trials.

Sincerely,

The Avidity Team