



September 10, 2025

Dear DMD Community,

Today, we are very pleased to share with you new positive data from the completed Phase 1/2 EXPLORE44® clinical trial and the ongoing EXPLORE44-OLE™ trial, designed to assess the safety and efficacy of our investigational therapy delpacibart zotadirsen (abbreviated as del-zota) in people living with Duchenne muscular dystrophy amenable to exon 44 skipping (DMD44).

The functional data show reversal of disease progression across key measures including the 4-Stair Climb, 10-Meter Run Walk Test, Time to Rise from Floor, Performance of Upper Limb, and stabilization of progression on the North Star Ambulatory Assessment when compared to baseline and natural history data of those living with DMD44.

The exploratory analysis also showed reduction in creatine kinase (CK) levels by greater than 80% compared to baseline. These changes were sustained at near normal levels over 16 months of follow-up. The analysis also showed increases of approximately 25% of normal in dystrophin production. EXPLORE44-OLE trial and del-zota continued to demonstrate supportive long-term safety and tolerability profile.

You can view our press release announcing the full results and safety data here: [Avidity Biosciences' Del-zota Demonstrated Reversal of Disease Progression Across Key Functional Endpoints in EXPLORE44® and EXPLORE44-OLE™ Phase 1/2 Trial in People Living with DMD44](#)

Del-zota is an investigational product and has not been approved by the FDA or any other regulatory authority for commercial use, and the safety and efficacy of del-zota has not been established.

COMMUNITY WEBINAR

Upcoming Webinar Hosted by CureDuchenne

To learn more about these findings, we invite you to join us for a webinar in partnership with CureDuchenne. The Avidity team will present the functional data, focusing on what these results mean for the DMD44 community.

Date: Thursday, September 18, 2025

Time: 2:00pm Pacific Time / 5:00pm Eastern Time

How to join: You can register to join the webinar via this link: [Webinar Registration - Zoom](#). A recording will be available on the CureDuchenne website within a week following the webinar.

We would like to extend our heartfelt gratitude to all participants in the EXPLORE44 development program, as well as their families, and our advocacy partners, and the investigators and their teams for their time and continued commitment. We share the urgency and the desire to bring a new treatment to the DMD community as quickly as possible. For the U.S. community, we remain on



track to submit a Biologics License Application (BLA) to the FDA by the end of 2025 for accelerated approval. We also continue to prepare for a phase 3 confirmatory study to support full global approval.

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. Please contact your doctor if you have any questions about del-zota, or the EXPLORE44 and EXPLORE44-OLE trials.

Sincerely,

The Avidity Team