Avidity Biosciences Managed Access Program (MAP) – Frequently Asked Questions

This document is intended to provide clarity and answer frequently asked questions from the Duchenne muscular dystrophy patient community about the Avidity Managed Access Program ("MAP") for the DMD44 investigational treatment delpacibart zotadirsen ("del-zota").

Del-zota is an investigational treatment (it has not been approved by the U.S. Food and Drug Administration (FDA)) for people living with Duchenne muscular dystrophy ***amenable to exon 44 skipping*** *(DMD44). Del-zota is designed to cause skipping of exon 44 in the dystrophin gene and is being studied to determine if it increases dystrophin, a protein that muscles need to stay strong. Del-zota has not been approved by the FDA, and its safety and efficacy have not been established.*

Important note: this information is current as of January 2026 and is for education and awareness only. For questions related to del-zota, please contact your doctor.

What is a Managed Access Program ("MAP")?

- A Managed Access Program (sometimes called Early Access or Expanded Access) is a way for some patients, in consultation with their doctor, to receive an investigational medicine before it is approved by the FDA. These programs are typically for serious diseases or conditions with no or limited treatment options.
- The MAP is designed to give access to *del-zota* for patients who meet the program requirements and whose doctor believes the patient may benefit from the investigational treatment while the medicine is under review by the FDA.

Is the MAP the same thing as a clinical trial?

- No. A MAP is **not** a clinical trial.
- While a clinical trial evaluates the safety and efficacy of a drug, the purpose of a MAP is to provide treatment for serious diseases or conditions according to specific requirements.
- Therefore, the process for access to a medicine under a MAP is typically different than that for a clinical trial.
- In the Avidity MAP, Avidity provides *del-zota,* and it is the responsibility of the doctor to administer the program and oversee all activities according to guidelines.

How do I get access to the MAP?

- Talk with your doctor about whether the MAP may be an option for you or your child.
- You can also visit clinicaltrials.gov to learn more about the program (https://clinicaltrials.gov/study/NCT07250737).

How do I know which sites are participating in the program?

- As sites enroll into the MAP, the site name(s) will be added to the clinicialtrials.gov website (https://clinicaltrials.gov/study/NCT07250737). Please note this process takes time, and sites will only be added after they are fully enrolled in the program.

Do I have to go to one of the sites listed on the clinicialtrials.gov website?

- No. The sites listed on the website are active sites (sites that have completed the enrollment process and are approved to administer *del-zota*). Your doctor can contact the program to learn more about potential site enrollment.

Am I eligible for MAP/what are the eligibility criteria?

- You can find eligibility criteria by visiting the clinicialtrals.gov website (https://clinicaltrials.gov/study/NCT07250737).
- You may qualify if you:
    - Permanently reside in the U.S. and have a U.S. primary health care provider
    - Are 6 years of age or older
    - Have a DMD diagnosis with confirmed dystrophin gene mutation amenable to exon 44 skipping
- This is not the full list of criteria. Please talk with your doctor to see if you may qualify.

Is there access for kids younger than 6 years old?

- No. The MAP is only available for participants 6 years or older at the time of signing up. This is because the safety of the drug in younger patients is not known.

Can patients who have received gene therapy participate in MAP?

- Some individuals who have previously received gene therapy may be eligible for the MAP. Additional requirements to participate will apply. Please talk with your doctor to learn if you qualify.

How long will it take to get the treatment once I first talk to my doctor about MAP?

- The timing varies from site to site and could take several months. MAPs must follow FDA regulations, which include specific processes for site approval and patient enrollment. In addition, your doctor needs to complete the required paperwork before treatment can begin. The best thing to do is stay in contact with your doctor.

Will there be a cost for me to participate in MAP?

- *Del-zota* is provided **at no cost** to patients enrolled in the MAP.
- Because MAP is not a clinical trial, other costs (that may be covered under a clinical trial) cannot be covered.
- Routine medical costs (such as lab tests) will be handled the same way as a regular doctor's visit and are typically billed through insurance.
- Travel costs cannot be provided, or reimbursed, by Avidity.

Does my doctor need to have an infusion center onsite?

- No. The infusion center does not need to be located at your doctor's office. Your doctor will decide the most appropriate place for treatment.

How is *del-zota* administered and how often is the dose?

- *Del-zota* is administered as an intravenous (IV) infusion (through a vein). Infusions take place **once every 6 weeks**.

Is a port necessary for infusion?

- No, a port is not required.

Is the MAP available for current EXPLORE44-OLE clinical trial participants?

- Yes. Participants in the EXPLORE44-OLE must complete the clinical trial before they are eligible to receive *del-zota* via the MAP. Participants should talk with their study doctor about MAP options as they get close to completing the trial.

If I'm on the clinical trial, do I have to get the MAP treatment at the clinical trial site?

- No. Participants are not required to receive MAP treatment at the same site as the clinical trial and may be able to receive treatment closer to home by a doctor who has enrolled in the MAP.

Is MAP available for people located outside the United States?

- Currently, MAP is only available to participants who are permanently residing in the U.S. and have a U.S. primary health care provider.

What should I do if I have additional questions?

- For information on the program, please talk with your doctor.