

## EXONDYS 51 (eteplirsen) FAQ

September 2025

- **What is EXONDYS 51?**
  - [EXONDYS 51 \(eteplirsen\)](#) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy in people who have a confirmed variant of the *DMD* gene that is amenable to exon 51 skipping. It is a once weekly infusion through an IV or port.
- **How does it work?**
  - EXONDYS 51 works by skipping additional segments of the dystrophin code called exons, shifting from an [out-of-frame deletion to an in-frame deletion](#). Typically, an in-frame deletion results in a smaller, but still functional, dystrophin protein. This shortened protein is expected to act in a similar way to normal dystrophin, and relieve some symptoms of Duchenne and hopefully result in a more mild progression.
- **How is EXONDYS 51 administered?**
  - EXONDYS 51 is administered once a week as an infusion through an IV or port. Infusions may be given at your neuromuscular clinic, an infusion center or at your home.
- **Who is eligible for EXONDYS 51?**
  - EXONDYS 51 is approved for people who have a deletion in the dystrophin gene that can be treated by skipping exon 51. If you know your/your child's genetic change (variant) is an exon deletion, PPMD's [Exon Deletion Tool](#) can help you understand if you/your child may be a candidate for EXONDYS 51.
  - If you are unsure of your child's genetic variant or have questions about eligibility, please reach out to your neuromuscular team.
- **Important things to know about EXONDYS 51:**
  - It's important to talk with your neuromuscular provider about risks, benefits, and precautions before starting any medications.
  - You can read the full prescribing label of EXONDYS 51 [here](#).
- **What are the possible side effects of EXONDYS 51?**
  - The most common side effects were vomiting, problems with balance and skin irritation. You can find more information on side effects [here](#).
  - If you/your child notices any side effects, contact your neuromuscular provider.
- **What precautions are needed while taking EXONDYS 51?**
  - It is important to watch for hypersensitivity reactions. If this occurs, seek medical treatment and contact your neuromuscular provider.
- **How does my child get access to EXONDYS 51?**
  - If you want to know if your child is eligible to receive EXONDYS 51, call your neuromuscular team to discuss whether EXONDYS 51 is right for your child.
  - With your consent and signature, your doctor will complete and submit the SareptaAssist Enrollment Form, which will begin the [SareptaAssist process](#). Your neuromuscular provider and Sarepta case manager will work together to obtain insurance approval.
  - Once approved, your case manager will work with the specialty pharmacy to start treatment access and coordinate delivery of the drug and treatment location

- Learn more about access & coverage resources for EXONDYS 51 [here](#).
- [Learn more about clinical trials of EXONDYS 51](#)