

## AMONDYS 45 (casimersen) FAQ

September 2025

- **What is AMONDYS 45?**
  - [AMONDYS 45 \(casimersen\)](#) is an antisense oligonucleotide for individuals with Duchenne muscular dystrophy who have a specific genetic variant amenable to exon 45 skipping. It is a once weekly infusion through an IV or port.
- **How does it work?**
  - AMONDYS 45 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 may relieve some symptoms of Duchenne and hopefully result in a more mild progression
- **How is AMONDYS 45 administered?**
  - AMONDYS 45 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 AMONDYS 45?**
  - AMONDYS 45 is [approved](#) for people who have a variant in the dystrophin gene that can be treated by skipping exon 45. 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 AMONDYS 45
  - 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 AMONDYS 45**
  - It's important to talk with your neuromuscular provider about risks, benefits, and precautions before starting any medications.
  - Your doctor may recommend urine and blood testing before starting treatment followed by urine testing every month and a blood test every 3 months to monitor your kidneys.
  - You can read the full prescribing label of AMONDYS 45 [here](#).
- **What are the possible side effects of AMONDYS 45?**
  - The most common side effects were upper respiratory tract infection, cough, fever, headache, joint pain and pain in mouth and throat. 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 AMONDYS 45?**
  - 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 AMONDYS 45?**
  - If you want to know if your child is eligible to receive AMONDYS 45, call your neuromuscular team to discuss whether AMONDYS 45 is right for your child.

- With your consent and signature, your doctor will complete and submit the SareptAssist Enrollment Form, which will begin the [SareptAssist 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 AMONDYS 45 [here](#).
- [Learn more about clinical trials of AMONDYS 45](#)