VILTEPSO (viltolarsen) FAQ

September 2025

- **What is VILTEPSO?**
  - VILTEPSO™ (viltolarsen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in people who have a confirmed variant of the *DMD* gene that is amenable to exon 53 skipping. It is a once weekly infusion through an IV or port.

- **How does it work?**
  - VILTEPSO 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 VILTEPSO administered?**
  - VILTEPSO 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 VILTEPSO?**
  - VILTEPSO is approved for people who have a variant in the dystrophin gene that can be treated by skipping exon 53. 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 VILTEPSO.
  - 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 VILTEPSO:**
  - 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 and during treatment to monitor your kidneys.
  - You can read the full prescribing label of VILTEPSO here.

- **What are the possible side effects of VILTEPSO?**
  - The most common side effects were upper respiratory tract infection, injection site reaction, cough, and fever (pyrexia)/
  - If you/your child notices any side effects, contact your neuromuscular provider.

- **What precautions are needed while taking VILTEPSO?**
  - 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 VILTEPSO?**
  - If you want to know if your child is eligible to receive VILTEPSO, call your neuromuscular team to discuss whether VILTEPSO is right for your child.

    - With your consent and signature, your doctor will complete and submit a patient start form to NS Support**.** Your neuromuscular provider and NS Support team will work together to obtain insurance approval.
    - Once approved, your neuromuscular team 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 VILTEPSO here**.**

- **Learn more about clinical trials of VILTEPSO**

- **Video Library**
    - Webinar: VILTEPSO - AN FDA Approved Treatment for Patients Amenable to Exon 53 Skipping
    - Webinar: NS Pharma Announcement of VILTEPSO (viltolarsen)