



GETTING STARTED

 **Viltepso**[™]
(viltolarsen) injection

Indication

VILTEPSO is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VILTEPSO. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Important Safety Information

In clinical studies, no patients experienced kidney toxicity during treatment with VILTEPSO. However, kidney toxicity from drugs like VILTEPSO may be possible. Your doctor may monitor the health of your kidneys before starting and during treatment with VILTEPSO.

Please see additional Important Safety Information throughout.

Whether you are a parent of a child with DMD or a patient yourself, you've made the decision to start treatment with VILTEPSO. Throughout the journey, you might have some questions regarding treatment. We hope the following questions and answers provide some helpful information for you. Always remember: your doctor is your primary source for information.

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ABOUT VILTEPSO

What is VILTEPSO?

VILTEPSO is a treatment for Duchenne muscular dystrophy (DMD) that uses exon-skipping technology to increase dystrophin, a key protein for supporting muscle health.

What is exon skipping?

Exon skipping is a method of treatment designed to skip over a specific part of a gene (called an exon) that is missing or mutated. This allows the body to make a shortened form of the dystrophin protein.

Important Safety Information

Common side effects include upper respiratory tract infection, injection site reaction, cough, and fever.

Please see Important Safety Information throughout.

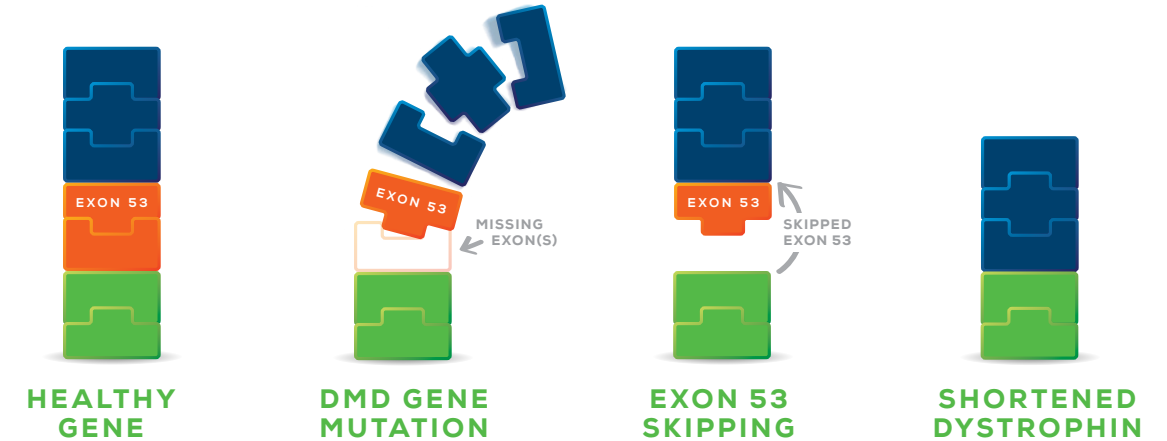
Who can take VILTEPSO?

VILTEPSO is approved for the treatment of DMD in patients amenable to exon 53 skipping.

How does VILTEPSO treat DMD?

DMD is caused by a mutation in the DMD gene that helps the body make dystrophin.

To better understand how VILTEPSO treats DMD, imagine genes as being made up of building blocks called exons



The DMD gene is made up of exons. These exons work together, much like the building blocks above—connecting in a specific way to create clear instructions on how to make full-length dystrophin protein.

A mutation or deletion in the DMD gene may impact the way the building blocks, or certain exons, fit together. As the blocks above illustrate, the exons aren't able to connect in the gene, which results in a lack of dystrophin production.

VILTEPSO is designed to skip over exon 53. In the image above, the orange block is skipped so the green block can fit next to the blue one. In DMD patients amenable to exon 53 skipping, this can result in the instructions for dystrophin production being communicated clearly.

As the blocks above show, when exon 53 is skipped, shortened dystrophin can be created. **VILTEPSO is proven to help the body make a shortened form of the dystrophin protein.**

For more information about VILTEPSO, see accompanying Product Information.





BENEFITS AND SAFETY

How does VILTEPSO impact dystrophin production?

VILTEPSO helps the body make more dystrophin, the protein needed to support the body's muscles. It has been shown to significantly increase dystrophin levels in children with DMD:

- After 20 to 24 weeks of treatment, children taking VILTEPSO (80 mg/kg/wk) showed an average dystrophin level of 5.9%, compared with 0.6% before starting treatment

What if my child has previously taken a different exon-skipping therapy?

Your doctor can answer any questions regarding your child's specific medications and VILTEPSO.

Are there any tests my child needs to do before starting VILTEPSO?

Please speak to your doctor before starting treatment with VILTEPSO. Patients with known renal function impairment should be closely monitored during treatment.

How will I know if VILTEPSO is working?

Your doctor is your number one resource when it comes to DMD and treatment response. He or she can evaluate symptoms and determine how your child is responding to treatment.

Symptoms may increase over time. Please document any of these noticeable changes and discuss with your doctor.

Setting goals is an important part of starting a treatment such as VILTEPSO. We recommend working with your doctor to set expectations and establish treatment goals.

There are other medications my child takes every day. Is it still OK to take VILTEPSO?

You should speak to your doctor about any questions about your child's medications.

What are the potential side effects of VILTEPSO?

As with any treatment, there are potential side effects. The most common side effects seen were upper respiratory tract infection, injection site reaction, cough, and fever. Speak with your doctor for medical advice about side effects.

For more information about VILTEPSO, see accompanying [Product Information](#).

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INFUSION PROCESS

How is VILTEPSO given?

VILTEPSO is administered as a once-weekly intravenous (IV) infusion that goes directly into the bloodstream through a small needle and tube. IV infusions allow for the immediate delivery of the drug into the body.

How long is each infusion?

The infusion lasts 60 minutes. But plan for some extra time before and after treatment in case you have questions for the nurse, or your child needs posttreatment observation.

How much medication is in each VILTEPSO dose?

Your doctor will calculate the weekly dosage based on your child's body weight. 80 milligrams of VILTEPSO is given for each kilogram (a kilogram is approximately 2.2 pounds) of your child's weight.

Where is the VILTEPSO infusion given?

VILTEPSO is administered by a healthcare professional at your home or at a treatment center. Your doctor or NS Support will be able to provide more details about these options.

Is the infusion painful?

It's possible that the individual receiving the infusion will feel pain during or after the injection. Discuss this with your child's doctor to determine the best plan.

Will my child require sedation during the infusion?

While sedation is not required, your doctor will determine if it will be necessary during the procedure. Your doctor is your primary resource when it comes to understanding the treatment procedure with VILTEPSO.

Who will monitor my child during the infusion?

Your healthcare team will monitor your child during the infusion—either at home or at the treatment center.

What happens if a scheduled treatment appointment is missed?

It's important to work with your child's doctor in the event of a missed treatment appointment. Your doctor can determine the next course of action per the VILTEPSO full Prescribing Information.

If a dose of VILTEPSO is missed, it should be administered as soon as possible after the scheduled time.

You can find more information about the VILTEPSO infusion process, and what to expect on treatment day, by reading the Infusion Overview that is included in the VILTEPSO Welcome Kit.

Important Safety Information

Common side effects include upper respiratory tract infection, injection site reaction, cough, and fever.

Please see Important Safety Information throughout.

For more information about VILTEPSO, see accompanying Product Information.





ADDITIONAL QUESTIONS

How can I find an available treatment center?

Please contact your doctor or NS Support to find a convenient treatment center.

How will I know if my health insurance will cover the VILTEPSO prescription?

NS Support can help you understand your current insurance benefits, or counsel you and your family on the possibility of adding or changing insurance coverage, as needed.

What resources are available to help me with insurance issues or financial concerns?

You can access NS Support, which can provide comprehensive care coordination and resources from NS Pharma.

NS Support will provide a personal case manager who will offer individualized care and support throughout the process by:

- Explaining insurance benefits and out-of-pocket cost support options
- Discussing alternative and supplemental sources of financial assistance
- Providing information about national and local advocacy organizations offering support for patients

For more information about VILTEPSO, see accompanying Product Information.



Please reach out to NS Support with any questions regarding treatment with VILTEPSO at **833-NSSUPRT** (833-677-8778), Monday through Friday, 8 AM to 8 PM ET.





NOTES

We hope you found this information about VILTEPSO useful.

We encourage you to refer to it as needed, regardless of where you and your child are on the treatment journey.

The following pages are for you to add any follow-up questions or concerns you may have for your doctor.





NOTES

Speak to your healthcare professional
for more information about VILTEPSO,
or visit [VILTEPSO.com](https://www.viltepsosupport.com)



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