Viltepso®
(viltolarsen) injection

MICHAEL – Age 14

ROLAND – Age 4

Treating Duchenne means hope to share meaningful moments

MASON – Age 11

DIEGO – Age 19

JORDAN – Age 15

These are real VILTEPSO patients and compensated spokespeople who have been taking VILTEPSO for at least 2 years.

Prescription data shows that most people taking VILTEPSO choose to remain on treatment. 92% of people who started VILTEPSO were still taking it one year later.

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

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What Is DMD?

Duchenne muscular dystrophy (DMD) is a rare genetic disease that results in muscles becoming damaged and weaker over time.

DMD is caused by a missing or mutated part of the gene that normally produces dystrophin

With a mutation or deletion in the DMD gene, the body produces unusable dystrophin that can't properly support muscle function

EARLY DIAGNOSIS IS KEY to helping manage progressive muscle weakness and functional decline in patients with DMD

For muscles to function properly,
they need a protein called
DYSTROPHIN

Important Safety Information (continued)

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

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VILTEPSO is proven to help the body make a shortened form of dystrophin protein

HEALTHY DMD GENE

The Duchenne muscular dystrophy (DMD) gene is made up of individual pieces called exons. These exons work together like building blocks to tell the body how to make a full-length dystrophin protein.

DMD GENE MUTATION

A *mutation or deletion in the DMD gene* may impact the way its exons fit together. As you can see here, exon 53 has lost its connecting partner, which prevents the body from making enough usable dystrophin to support skeletal muscles.

EXON 53 SKIPPING

In DMD patients amenable to exon 53 skipping, *VILTEPSO is designed to skip over exon 53.* In this case, it skips over the orange block (exon 53) so that the green block can fit next to the blue one.

SHORTENED DYSTROPHIN

By skipping exon 53, *VILTEPSO helps the body make a shortened but partially functional form of dystrophin protein.*

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In a clinical study, VILTEPSO significantly increased dystrophin production

VILTEPSO was studied in 16 ambulatory (walking) boys ages 4 to less than 10 years who were receiving a stable dose of corticosteroids for at least 3 months.

In this graph, their average dystrophin levels at week 25 of VILTEPSO treatment are compared with their average dystrophin levels before treatment.

P=0.01 normalized to myosin heavy chain

These significant increases in dystrophin production with VILTEPSO were identified by a method called western blot and verified by a highly sensitive measuring technique known as mass spectrometry.

100% of patients showed an increase in dystrophin levels with VILTEPSO†

†After 24 weeks, mean increase in dystrophin expression was nearly 6% of normal with VILTEPSO (80 mg/kg/wk) vs 0.6% at baseline (n=8).

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In the same clinical study, additional results included evidence of dystrophin production and timed motor function tests

Secondary endpoint*	Baseline (n=8)	Week 25 VILTEPSO (n=8)
Exon 53 skipping efficiency	0.0%	43.9% *P*=0.0001
Dystrophin production	0.6%	4.2% *P*=0.03
Dystrophin localization	1.8%	34.8% *P*=0.0026

*Exon 53 skipping efficiency assessed by RT-PCR. Mean dystrophin levels assessed by mass spectrometry. Dystrophin localization assessed by immunofluorescence staining.

RT-PCR=reverse transcriptase-polymerase chain reaction.

Functional tests were compared to Duchenne natural history study (DNHS) data as the control group rather than to placebo. Functional data are not in the US Prescribing Information.

Secondary endpoint†	DNHS-mean change from baseline at week 25 (n=65)	VILTEPSO-mean change from baseline at week 25 (n=8)
Time to stand		
(seconds)	0.66	-0.44

†Control subjects were matched for age and corticosteroids. Negative time means less time; positive time means more time.

Important Safety Information (continued)

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Safety profile evaluated in two 24-week clinical studies

Adverse reactions reported in ≥10% of people with DMD treated with VILTEPSO 80 mg/kg once weekly

Adverse reaction	VILTEPSO (80 mg/kg once weekly) (N=16); n (%)
Upper respiratory tract infection*	10 (63%)
Injection site reaction†	4 (25%)
Cough	3 (19%)
Pyrexia	3 (19%)
Contusion	2 (13%)
Arthralgia	2 (13%)
Diarrhea	2 (13%)
Vomiting	2 (13%)
Abdominal pain	2 (13%)
Ejection fraction decreased	2 (13%)
Urticaria	2 (13%)

*Upper respiratory tract infection includes the following terms: upper respiratory tract infection, nasopharyngitis, and rhinorrhea.

†Injection site reaction includes the following terms: injection site bruising, injection site erythema, injection site reaction, and injection site swelling.

No patients in the clinical trial discontinued treatment as a result of treatment-related Serious Adverse Events (SAEs)

Every person who took VILTEPSO in the 24-week study chose to continue taking VILTEPSO in the open-label follow-up study that lasted nearly four years.

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Meet real people taking VILTEPSO

Scan to see their full stories with videos on VILTEPSO.com

These stories describe unique experiences with VILTEPSO and are not intended to represent the average patient's response. Ask your doctor if VILTEPSO would be right for you or your loved one. Individual patient results with VILTEPSO may vary.

Meet Jordan

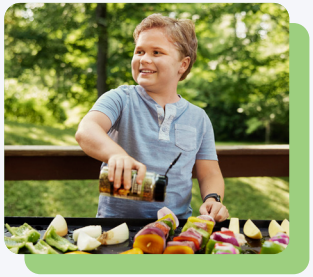
On therapy for over 7 years

Jordan (15) is a real VILTEPSO patient and compensated spokesperson.

When did Jordan start taking VILTEPSO?

Laura, Jordan's mom

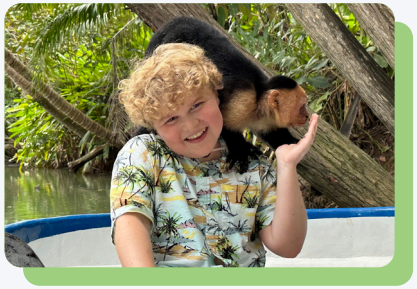
"Jordan started VILTEPSO as part of a clinical trial when he was almost 8 years old. Now, he's 15 years old and has had over 400 infusions!"



If you could say one thing to someone with Duchenne, what would it be?

Jordan

"Having Duchenne doesn't have to stop you from doing the things that you love to do. Sometimes you might just have to do things a little differently or ask friends and family for help."



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Meet Roland

On therapy for over 2 years

Roland (4) is a real VILTEPSO patient and compensated spokesperson.

What was Roland being diagnosed with Duchenne like?

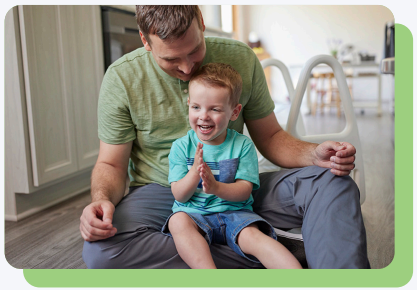
Amanda, Roland's mom

"We found out Roland had Duchenne just before his second birthday. I just have to work harder, play harder, and love him harder."

Does Roland taking VILTEPSO give you hope?

Nick, Roland's dad

"VILTEPSO gives Roland the opportunity to make some dystrophin. So yes, Roland taking VILTEPSO gives us hope."



Meet Mason

On therapy for over 3 years

Mason (11) is a real VILTEPSO patient and compensated spokesperson.

What are VILTEPSO home infusions like?

Rebecca, Mason's mom

"It's been really great having a nurse here at home, and not having to go to an infusion center and see somebody new every time."

If you could say one thing to another caregiver of a child with Duchenne, what would it be?

Rebecca, Mason's mom

"You're stronger than you know. You don't realize when you hear that diagnosis how many hopes and dreams you need to let go. But you also recalibrate and say, 'How important were those things?' It really helps to put what's most important into focus."

Important Safety Information (continued)

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Meet Michael

On therapy for over 2 years

Michael (14) is a real VILTEPSO patient and compensated spokesperson.

How would you say Michael is doing now?

Nadia, Michael's mom

"Long story short, we are more optimistic right now about Michael's future than we were 7 years ago, when he was just diagnosed."

What's it like taking VILTEPSO?

Michael

"Taking VILTEPSO at home is great because we don't have to drive two hours each way. And I can get more time to do things I like to do, like play music."

Meet Diego

On therapy for over 3 years

Diego (19) is a real VILTEPSO patient and compensated spokesperson.

How would you say Diego is doing now?

Leslie, Diego's mom

"I know many variables come into play with Duchenne, but I can see that right now, Diego is doing great. And taking VILTEPSO helps us keep up that hope that we all need."

If you could say one thing to someone with Duchenne, what would it be?

Diego

"I would say that your condition does not define who you are. Your strength and resilience make you who you are."

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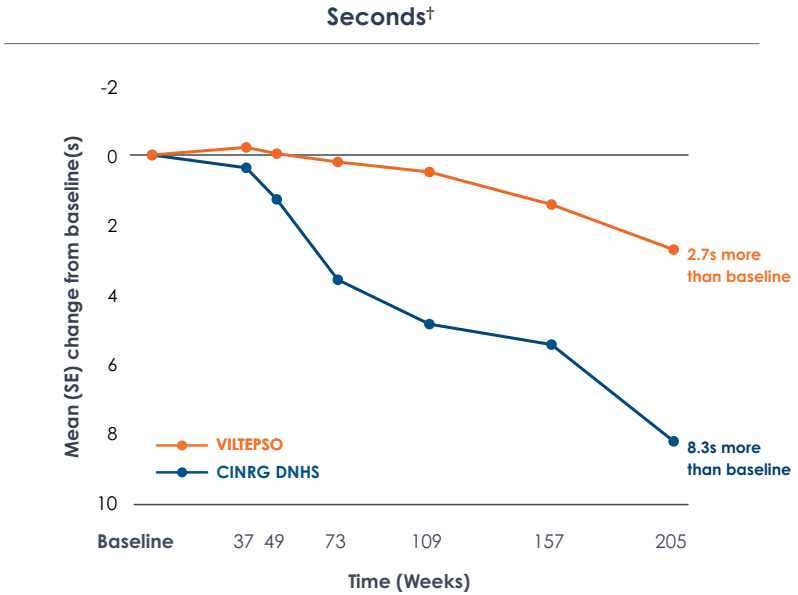
An extended view of patients on VILTEPSO over 4 years

Time to stand over 4 years*

With VILTEPSO, the mean change from baseline at week 205 was 2.7 seconds, and in the CINRG group, the mean change from baseline at week 205 was 8.3 seconds.

Time to stand measures the amount of time it takes for a DMD patient to go from lying on their back to standing.



*The control subjects for this trial were matched for age, ambulatory status, corticosteroid use, and geographic location from the CINRG DNHS registry.
†Negative time means less time; positive time means more time.

CINRG=Cooperative International Neuromuscular Research Group.
DNHS=Duchenne Natural History Study.

Functional tests were compared to Duchenne natural history data as the control group rather than to placebo. Functional data are not in the US Prescribing Information.

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Four-Year Safety Data

Safety assessment for open-label, 4-year extension study data

	VILTEPSO participants		
Participants with:	**40 mg/kg/wk n=8**	**80 mg/kg/wk n=8**	**Total N=16**
Any TEAE, n (%)	8 (100)	8 (100)	16 (100)
Any drug-related TEAE, n (%)	0	1 (13)	1 (6)
Any serious treatment-related AE, n (%)	0	0	0
Study drug discontinuation due to TEAE, n (%)	0	0	0
Death, n (%)	0	0	0

AE=adverse event; TEAE=treatment-emergent AE; wk=week.

No patients discontinued the study as a result of treatment-related Serious Adverse Events (SAEs)

Duchenne muscular dystrophy (DMD) is caused by not having enough dystrophin. VILTEPSO is an exon-skipping therapy that has been granted accelerated approval based on its demonstrated increase in dystrophin in patients with DMD amenable to exon 53 skipping. Results from a Phase 3 confirmatory study of VILTEPSO have been received and are undergoing analysis and discussion with the FDA.

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Since its FDA-accelerated approval in August 2020, people being treated with VILTEPSO have completed 94% of their weekly infusions.

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Taking VILTEPSO

VILTEPSO is a once-weekly intravenous (IV) infusion that can be given by a healthcare professional at your home or at a treatment center.

Here are a few questions you may have:

Q: What is an infusion?

A: An IV infusion goes into the patient's bloodstream through a small needle and tube. It is a **FAST** way to get medication directly into the body.

Q: How much medication is in each VILTEPSO dose?

A: Your healthcare provider will calculate the dose based on the patient's body weight. **80 MILLIGRAMS** of VILTEPSO is given for each kilogram (a kilogram is approximately 2.2 pounds) of your child's weight per week.

Q: How long is the infusion?

A: 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 post-treatment observation.

Viltepso®
(viltolarsen) injection

NS Support®
NS PHARMA ACCESS SOLUTIONS

Providing personalized access support and customized resources

Our experienced, knowledgeable team at NS Support is dedicated to assisting patients, their caregivers, and healthcare professionals throughout the patient journey to create a smooth path to treatment. We're committed to being here for you every step of the way.

SUPPORT SERVICES

- Individualized, caring support and resources throughout the patient journey
- Help with understanding insurance coverage for VILTEPSO
- **Co-pay Assistance Program**—eligible patients may qualify for savings on their deductible, co-pay, and coinsurance for their medication costs for VILTEPSO
- **Patient Assistance Program**—provides medication free of charge to patients who meet eligibility requirements

Eligible patients with commercial insurance coverage for treatment are automatically enrolled in the **Co-pay Assistance Program.**

- Savings on their deductible, co-pay, and coinsurance related to their medication costs
- Automatic re-enrollment for the next calendar year

Visit VILTEPSO.com/support for more information on NS Support, applications, and forms with details on eligibility requirements.

PATIENT SUPPORT WITH A PERSONAL TOUCH

CONNECT WITH NS SUPPORT — **833-NSSUPRT (833-677-8778)**

Monday–Friday, 8 AM–8 PM ET

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Talk to a doctor about VILTEPSO

VILTEPSO is effective at increasing dystrophin, a vital protein that supports muscle function.

Sign up for updates at **VILTEPSO.COM**

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NS Pharma

Viltepso® (viltolarsen) injection

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