Viltepso®
(viltolarsen) injection

Treating Duchenne means hope to share meaningful moments

Jordan (14 years old), a real VILTEPSO patient and compensated spokesperson, and his mother, Laura

VILTEPSO increases dystrophin, a key protein for supporting muscle health.*

*In a clinical study, 100% of patients taking VILTEPSO showed an increase in dystrophin levels. Average dystrophin level increase was nearly 6% at week 25 of treatment.

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 Important Safety Information throughout and see accompanying Product 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

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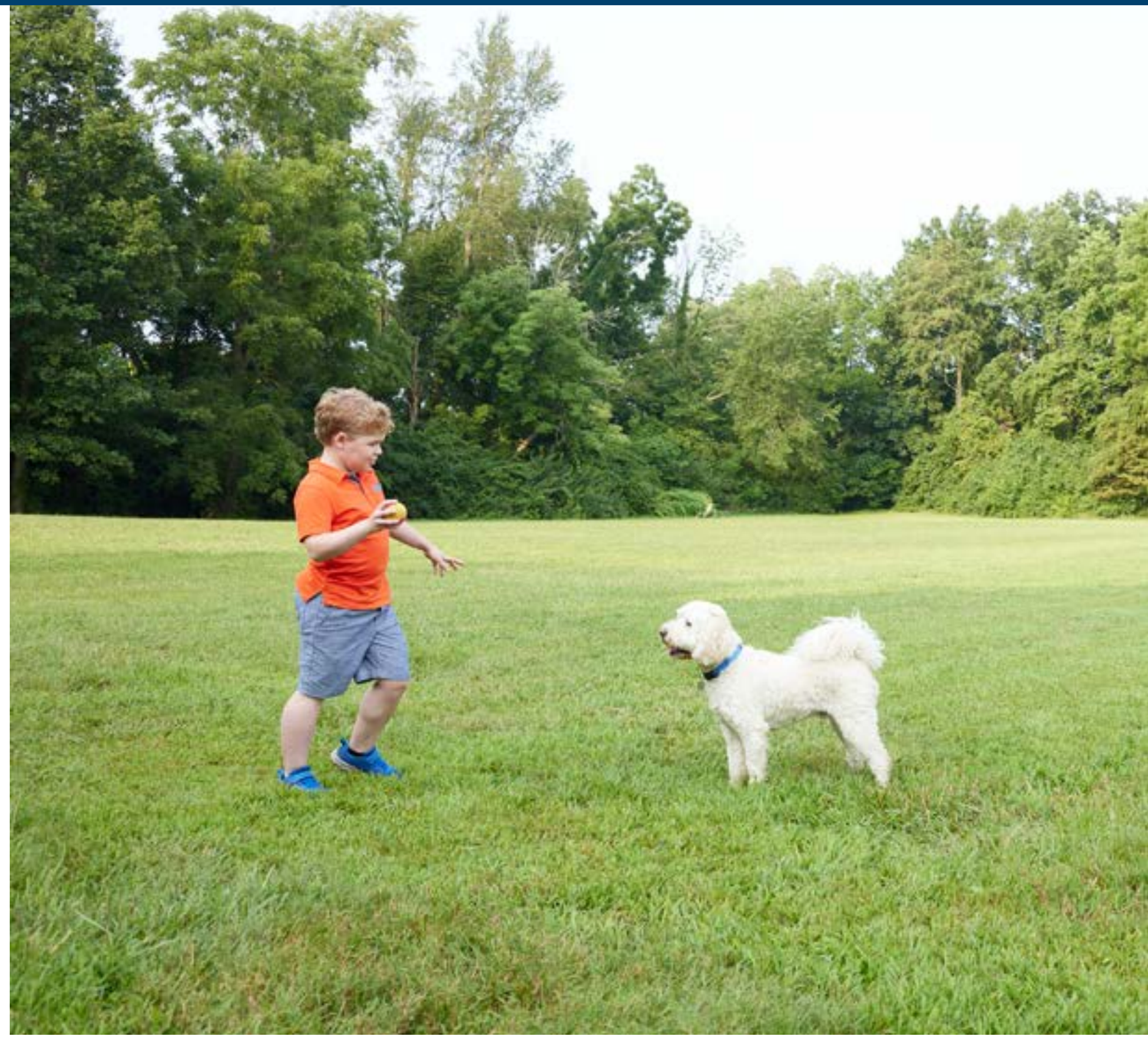

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VILTEPSO is proven to help the body make a shortened form of dystrophin protein

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 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 above, 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 usable form of dystrophin protein*.

Important Safety Information (continued)

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

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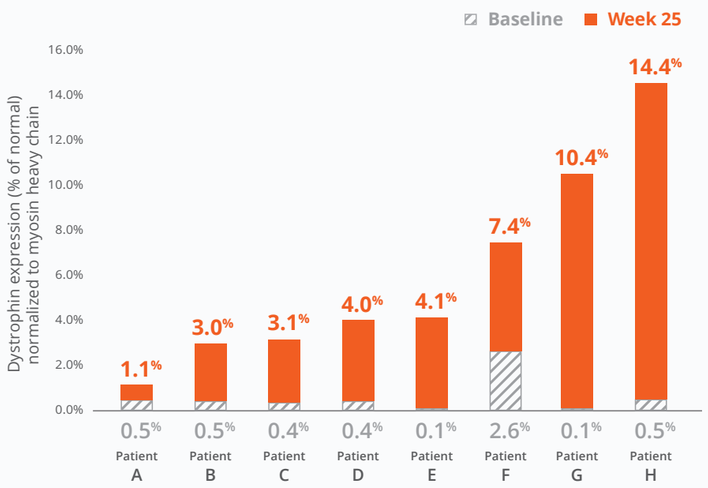
In a clinical study, 100% of people showed an increase in dystrophin levels with VILTEPSO



VILTEPSO significantly increased dystrophin production*

*88% (7 out of 8 people) showed increases of ~3% or higher, measured at week 25 of treatment. 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.

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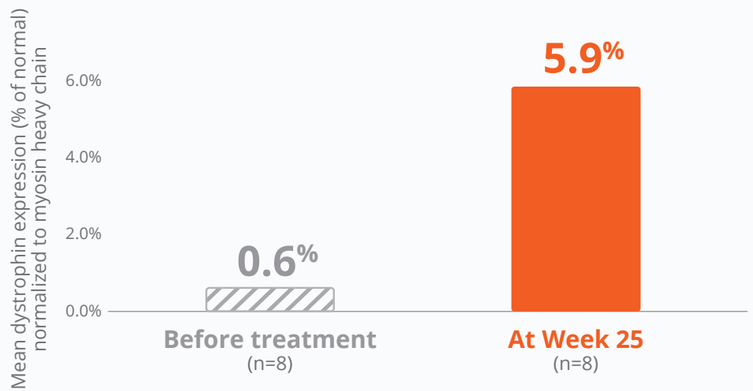
VILTEPSO increased dystrophin levels to nearly 6% of normal



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.

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In the same clinical study, additional results included evidence of dystrophin production and timed muscle 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 (DNHS) data as the control group rather than to placebo. Definitive conclusions should not be drawn. 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
Time to climb 4 stairs		
(seconds)	0.15	0.00
Time to run/walk 10 meters		
(seconds)	0.08	-0.66
6 minute walk test		
meters	-65.3	44.0
North Star Ambulatory Assessment (NSAA)	-1.1	1.1

†Control subjects were matched for age and corticosteroids. Negative time means less time; positive time means more time. Negative distance means less distance traveled; positive distance means greater distance traveled. Negative NSAA means lower score compared to baseline; positive NSAA means higher score compared to baseline.

Important Safety Information (continued)

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

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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.

Jordan and his father, Jeff

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

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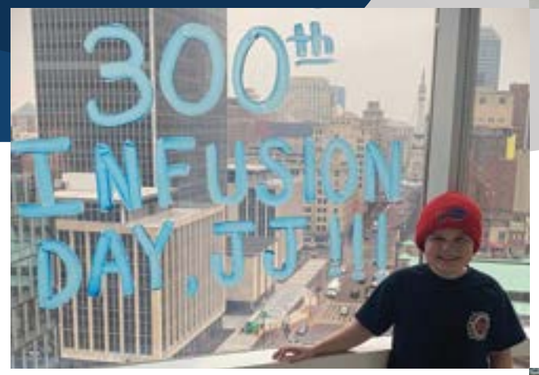
Meet Jordan



Each patient's experience is unique. Ask your doctor if VILTEPSO would be right for you or your loved one.

Jordan is a 14-year-old boy who has been taking VILTEPSO for six years. He is a real VILTEPSO patient and a compensated spokesperson. Here Jordan's mom, Laura, answers a few of our questions about his journey with VILTEPSO.

When did Jordan start VILTEPSO?

Laura: In 2017, when Jordan was almost 8 years old, he started treatment with VILTEPSO as part of a clinical trial. Now, he is 14 years old and has had over 300 infusions!

What does Jordan like to do for fun?

Laura: He likes walking our dogs, visiting the zoo, and going to different museums. He grows his peppers in our garden and really loves cooking, especially when he gets to use his peppers! He enjoys playing board games with us as a family and video games with his friends. Lately, he's really gotten into 3D printing and robotics.

This describes one patient's experience with VILTEPSO and is not intended to represent the average patient's response. Individual patient results with VILTEPSO may vary.

Important Safety Information (continued)

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

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Muscle function data

An extended view of patients on VILTEPSO over 4 years

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

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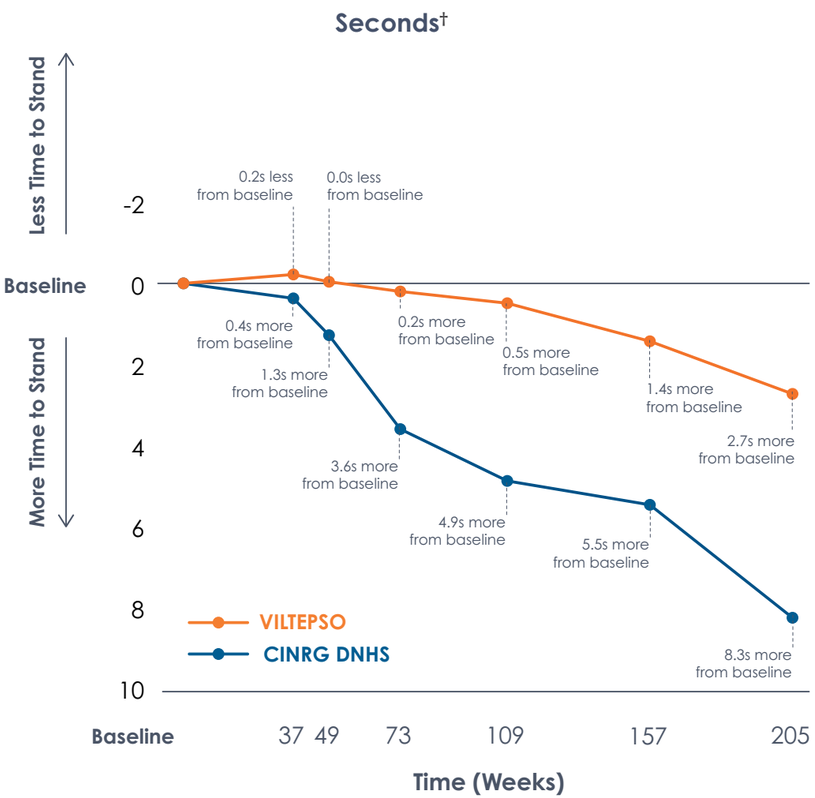
Time to stand over 4 years*

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

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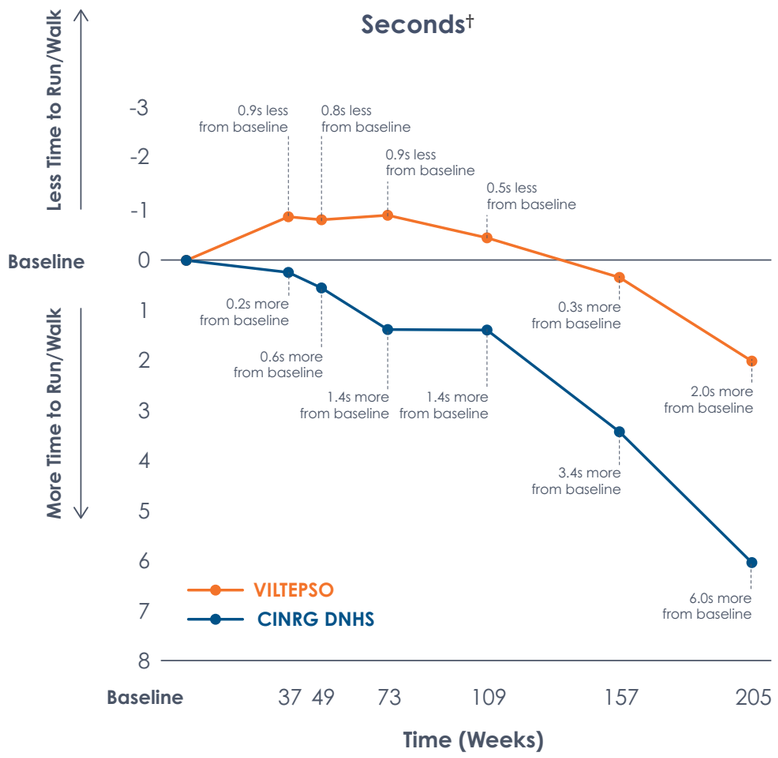
Time to run/walk 10 meters over 4 years*

With VILTEPSO, the mean change from baseline at week 205 was 2.0 seconds and in the CINRG group the mean change from baseline at week 205 was 6.0 seconds

Time to run/walk 10 meters measures the amount of time it takes for a DMD patient to run or walk 10 meters.



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Important Safety Information (continued)

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

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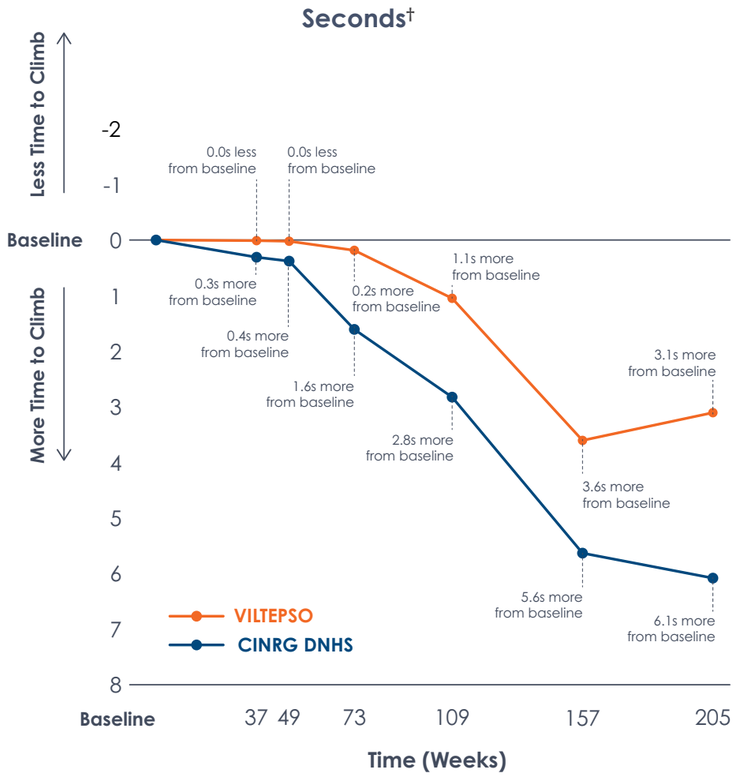
Time to climb four stairs over 4 years*

With VILTEPSO, the mean change from baseline at week 205 was 3.1 seconds and in the CINRG group the mean change from baseline at week 205 was 6.1 seconds

Time to climb four stairs measures the amount of time it takes for a DMD patient to climb four stairs.



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Four-Year Safety Data

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

	Viltolarsen 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)

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 other 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.

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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**—help for uninsured patients in financial need

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 and an application 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.

In a clinical study, **100% of patients taking VILTEPSO** showed an increase in dystrophin levels. Average dystrophin level **increase was nearly 6%** at week 25 of treatment.

SIGN UP FOR UPDATES

VILTEPSO.COM

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

You are encouraged to report adverse events related to VILTEPSO. To do so, or for general inquiries, please call NS Pharma Medical Information at 1-866-NSPHARM (1-866-677-4276).

For more information about VILTEPSO, see full Prescribing Information.

Please see Important Safety Information throughout and see accompanying Product Information.

NS Pharma

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