Viltepso®
(viltolarsen) injection

A treatment option for people with Duchenne could mean hope to share meaningful moments

VILTEPSO is for people with Duchenne muscular dystrophy amenable to exon 53 skipping[1]

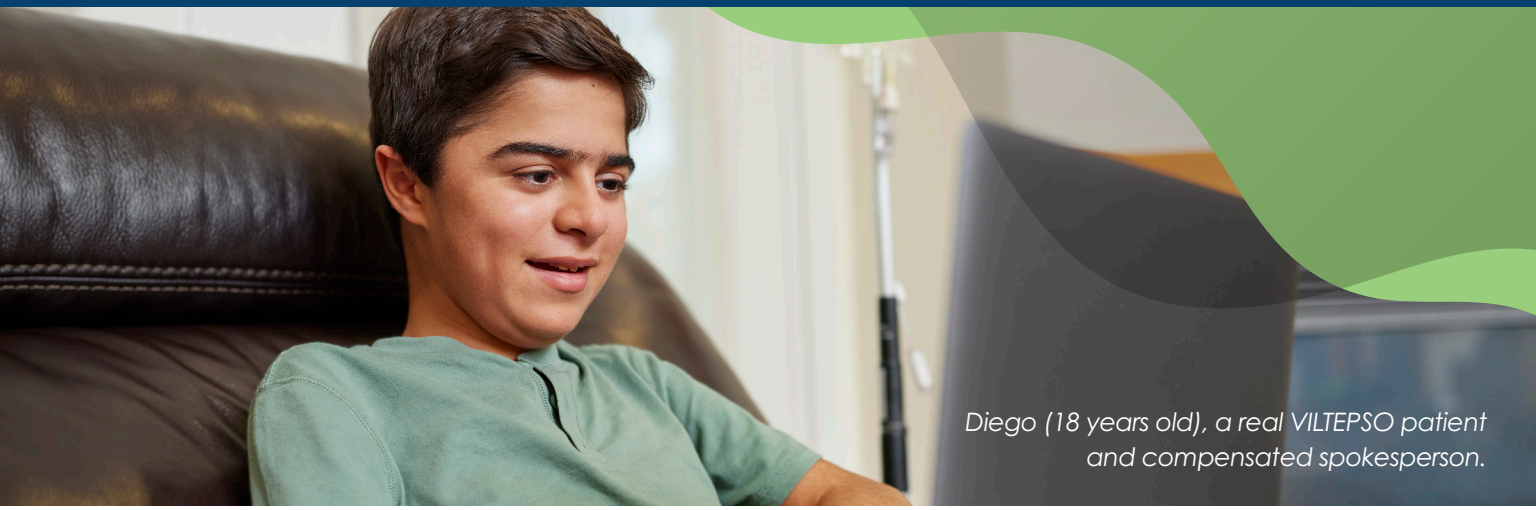
Indication

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

Warnings and Precautions: Kidney toxicity was observed in animals who received viltolarsen. Although kidney toxicity was not observed in the clinical studies with VILTEPSO, the clinical experience with VILTEPSO is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VILTEPSO. Serum creatinine may not be a reliable measure of kidney function in DMD patients.

Please see Important Safety Information throughout. For additional information about VILTEPSO, see accompanying full Prescribing Information.



Diego (18 years old), a real VILTEPSO patient and compensated spokesperson.

VILTEPSO is for DMD patients with a confirmed mutation that is amenable to exon 53 skipping[1]

VILTEPSO is designed to skip **exon 53 of the dystrophin pre-mRNA,** resulting in the production of a shortened dystrophin protein containing essential functional portions[1]

Exon-skipping is designed to correct an out-of-frame variant and **enables the expression of a shorter dystrophin protein**[2]

VILTEPSO increased dystrophin levels from baseline after 20 to 24 weeks of treatment in **100% of patients** (see study details on page 3) during clinical trials as measured by western blot, the laboratory standard for protein detection[1]

SAFETY PROFILE

evaluated in two 24-week clinical studies[1]

Infused over

1 HOUR

by a healthcare professional, at home or at a treatment center, at a recommended weekly dose of 80 mg/kg[1]

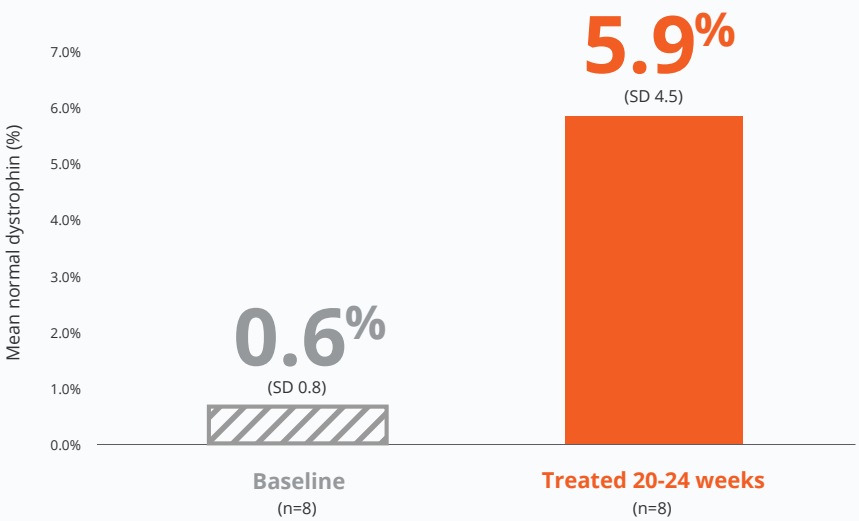
Important Safety Information (continued)

Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VILTEPSO. Consider also measuring glomerular filtration rate before starting VILTEPSO. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months.

VILTEPSO provided significant improvements in dystrophin expression[1,3]

The efficacy of 20-24 weeks of VILTEPSO was evaluated in ambulant males aged 4 to <10 years.[1,3]

Mean increase in dystrophin expression to nearly 6% of normal with VILTEPSO (80 mg/kg/wk) vs 0.6% at baseline[1,3]



- Efficacy was assessed by validated western blot (*P*=0.01 normalized to myosin heavy chain) based on the change from baseline in dystrophin protein level, measured as percentage of the dystrophin level in healthy subjects at week 25[1]
- Mean change in dystrophin was 5.3% (SD 4.5) of normal levels (p=0.01)[1]
  - Median change from baseline was 3.8%[1]

DYSTROPHIN WHERE IT MATTERS: Immunofluorescence staining showed VILTEPSO-induced increases in dystrophin levels were correctly localized to the muscle cell membrane, where dystrophin is needed to support muscle health.[1]

Important Safety Information (continued)

Urine should be free of excreted VILTEPSO for monitoring of urine protein. Obtain urine either prior to VILTEPSO infusion, or at least 48 hours after the most recent infusion. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, which has the potential to generate a false positive result due to cross reaction with any VILTEPSO in the urine. If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Please see Important Safety Information throughout. For additional information about VILTEPSO, see accompanying full Prescribing Information.

Safety profile evaluated in two 24-week clinical studies[1]

Adverse reactions reported in ≥10% of DMD patients treated with VILTEPSO 80 mg/kg once weekly[1]

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 treatment-related SAEs, drug-related TEAEs, discontinuations, or deaths occurred.[3,4]

SAE=serious adverse event; TEAE=treatment-emergent adverse event.

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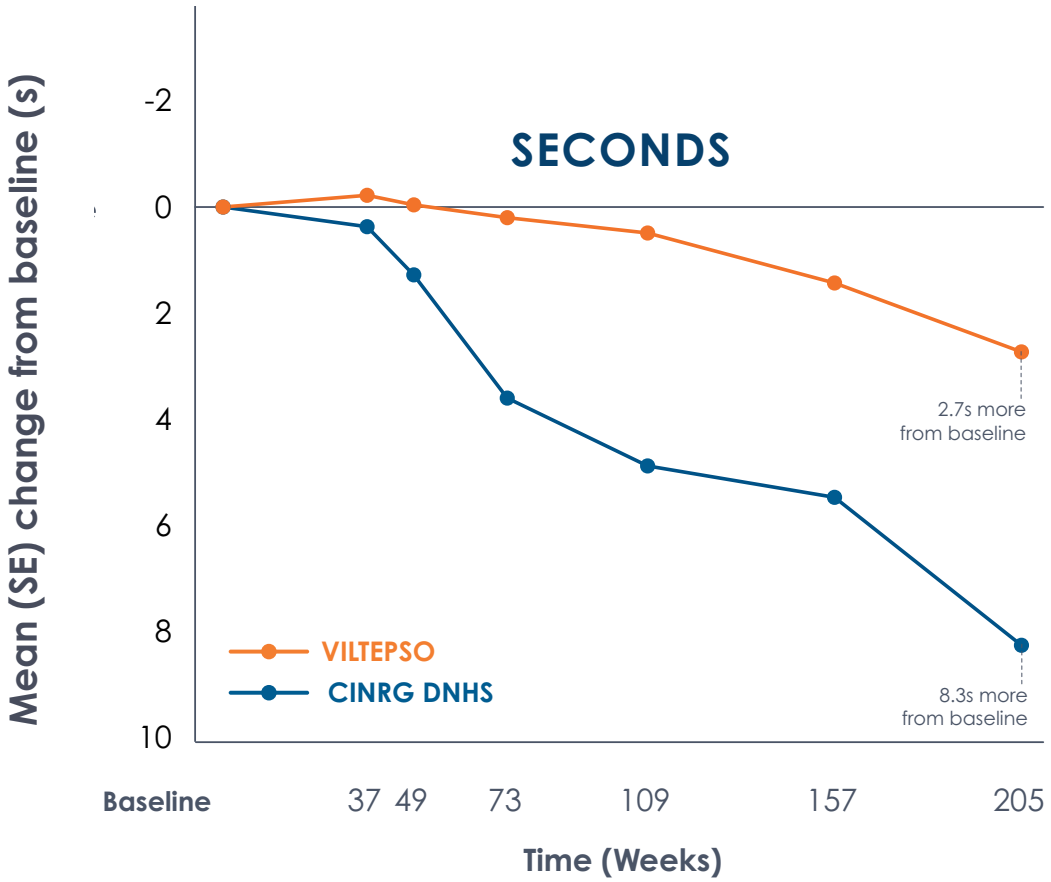
Motor function data from a 4-year, open-label extension study[5]

Time to stand over 205 weeks*



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.

*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.

Important Safety Information (continued)

Adverse Reactions: The most common adverse reactions include upper respiratory tract infection, injection site reaction, cough, and pyrexia.

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Safety assessment for open-label, 4-year extension study data[5]

	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; no patients died;
3 patients had SAEs, none of which were considered related to study drug.

References: 1. Viltepso [prescribing information]. Paramus, NJ: NS Pharma, Inc.; 2021. **2.** Watanabe N, Nagata T, Satou Y, et al. *Mol Ther Nucleic Acids*. 2018;13:442-449. **3.** Clemens PR, Rao VK, Connolly AM, et al; for the CINRG DNHS Investigators. *JAMA Neurol.* 2020;77(8):982-991. **4.** Komaki H, Takeshima Y, Matsumura T, et al. *Ann Clin Transl Neurol*. 2020;7(12):2393-2408. **5.** Clemens PR, Rao VK, Connolly AM, et al; J Neuromuscul Dis. 2023;10(3):439-447.

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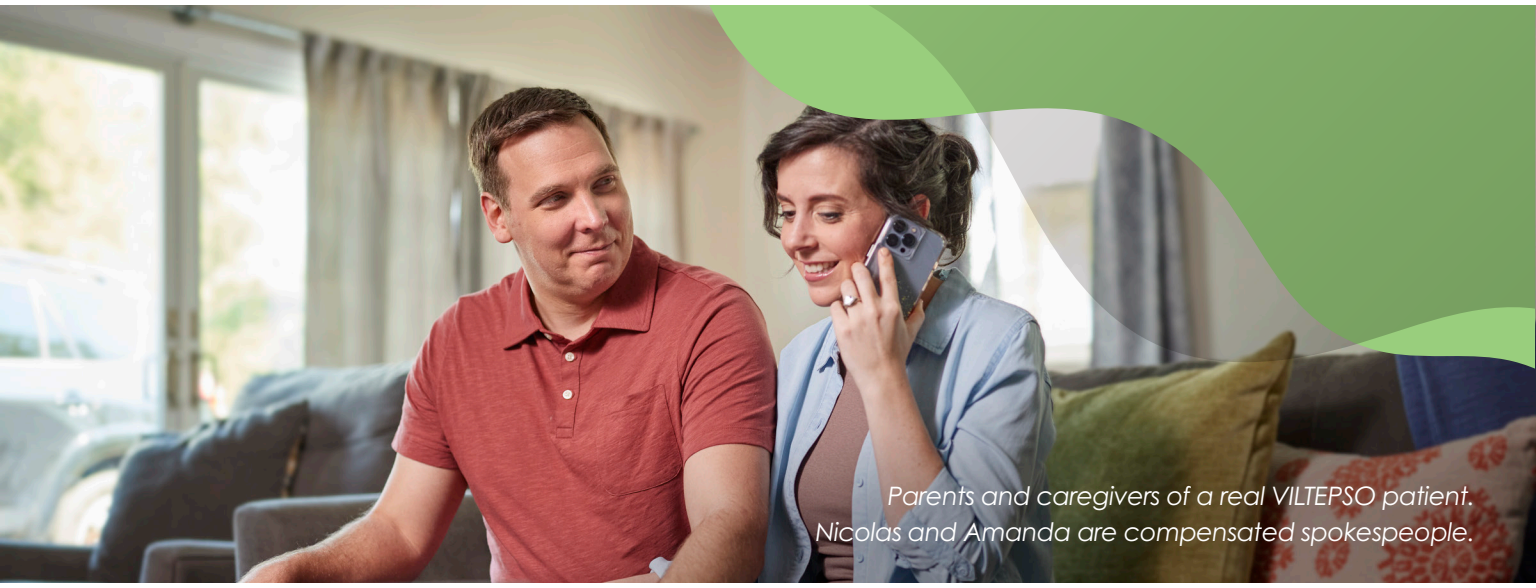

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VILTEPSO increased dystrophin levels in 100% of patients during clinical trials[1]

**Mean increase in dystrophin expression to nearly 6% of normal with VILTEPSO (80 mg/kg/wk) vs 0.6% at baseline.[1,3] Please see the study details listed on page 3.*

See more efficacy & safety data on **VILTEPSO.COM**

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Important Safety Information (continued)

To report an adverse event, or for general inquiries, please call NS Pharma Medical Information at 1-866-NSPHARM (1-866-677-4276).

Please see Important Safety Information throughout. For additional information about VILTEPSO, see accompanying full Prescribing Information.

NS Pharma