Viltepso®
(viltolarsen) injection

Treating Duchenne means hope to share meaningful moments

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


MICHAEL – Age 14


ROLAND – Age 4


MASON – Age 11


DIEGO – Age 19


JORDAN – Age 15

The individuals shown here are real VILTEPSO patients and compensated spokespeople who have been on treatment for at least 2 years. Each individual's experience with VILTEPSO is unique and not intended to be indicative of every patient's experience.

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.

These are real patients on VILTEPSO

Since VILTEPSO became commercially available in August 2020, VILTEPSO treated patients have received their weekly infusions at a rate of 93%.[2,3]

ROLAND
*Age **4**, on therapy for **2** years*

Diagnosed just before the age of 2, he was able to start VILTEPSO about 6 months later.

MICHAEL
*Age **14**, on therapy for **2** years*

His parents are more optimistic now about his future than when he was diagnosed at age 5.

MASON
*Age **11**, on therapy for **3** years*

His mom is pleased he gets his weekly infusions from the same nurse in their home.

These are real VILTEPSO patients and compensated spokespeople. Each individual's experience with VILTEPSO is unique and is not intended to be indicative of every patient's experience.

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.

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Most VILTEPSO patients choose to stay on treatment[3]

Real-world data from clinical practice shows that 86% of eligible patients remained on VILTEPSO treatment after 12 months.[3]

DIEGO
*Age **19**, on therapy for **3** years*

Moved to the U.S. with his mom so he could have better access to treatments like VILTEPSO.

JORDAN
*Age **15**, on therapy for **6** years*

*Started VILTEPSO as part of a clinical trial, and has since had over **400** weekly infusions.*

See their stories on VILTEPSO.com/patient/stories

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

VILTEPSO corrects out-of-frame mutations by skipping exon 53 of the dystrophin pre-mRNA.*,[1]

Exon-skipping therapy is designed to produce a shortened dystrophin protein containing essential functional portions.[4,5]

During clinical trials, VILTEPSO increased dystrophin in 100% of patients, with a mean increase to nearly 6% of normal with VILTEPSO (80 mg/kg/wk) vs. 0.6% at baseline.[1]

*Inside each muscle cell, DNA provides the genetic information to create dystrophin. The first step in this process is the formation of pre-mRNA from DNA. The pre-mRNA is further processed into mRNA, which is then translated into a protein.[6]

SAFETY PROFILE

evaluated in two 24-week studies, two 48-week studies, and a 4-year open-label extension study.[1,7,8,9]

Infused over

1 HOUR

by a healthcare professional, at home or at a treatment center.[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.

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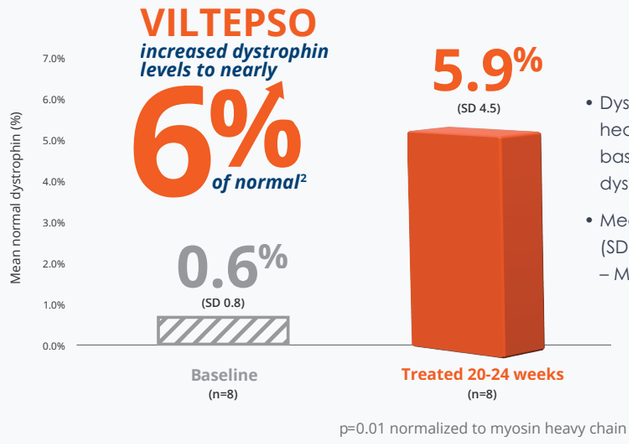
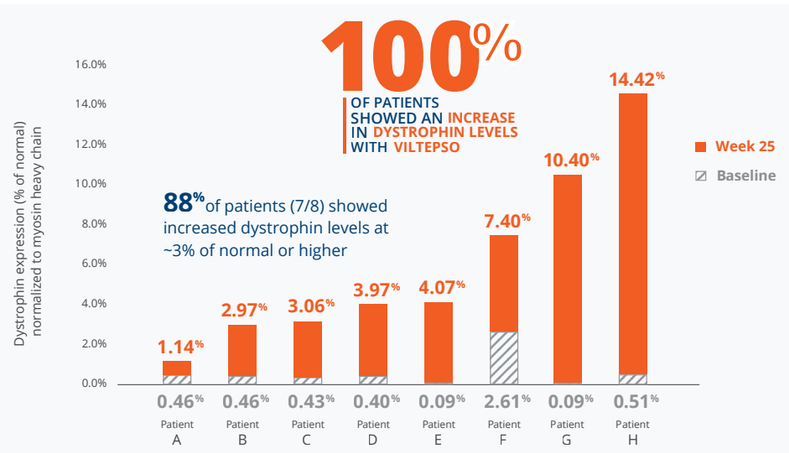
VILTEPSO significantly improved dystrophin production[1]

In the primary phase 2 study, the efficacy of 20-24 weeks of VILTEPSO treatment (80 mg/kg/wk) was evaluated in ambulatory males aged 4 to less than 10 years.[1]



- Dystrophin protein level (normalized to myosin heavy chain) was based on the change from baseline, measured as percentage of the dystrophin level in healthy subjects, at week 25[1,3,10]
- 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]

The statistically significant increase in dystrophin expression was measured by western blot analysis, which is a validated, highly sensitive, and reproducible methodology.[1,3,10]



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Secondary endpoint results included a motor function test[3]

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

†The control subjects for this trial were matched for age and corticosteroids 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.

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

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

Adverse reaction	VILTEPSO (80 mg/kg/wk) (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.

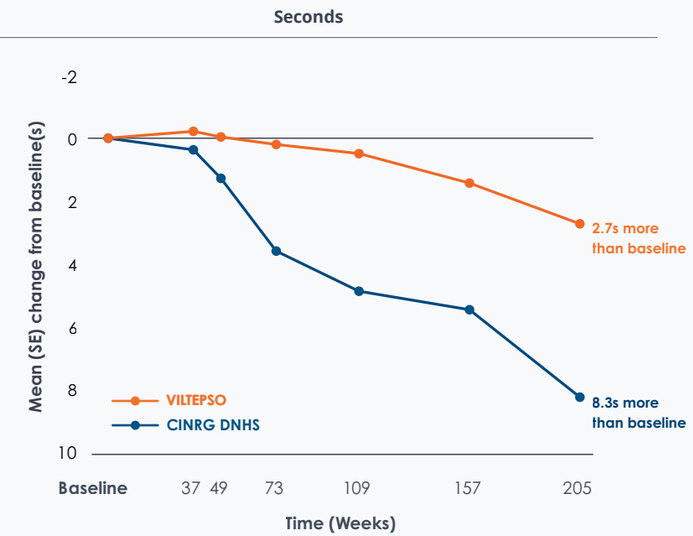
SAE=serious adverse event;
TEAE=treatment-emergent adverse event.

No treatment-related SAEs, drug-related TEAEs, discontinuations, or deaths occurred.[10]

Long-term, 4-year, open-label extension study: functional assessment data[7]

After completing the 24-week primary study, patients were given the option to enroll in an additional 192-week open-label extension study, in which efficacy assessments were conducted every 12 weeks.[7,11] **All patients in the primary study chose to continue VILTEPSO for the entirety of the long-term extension study.**[7]

Time to stand over 205 weeks*,[7]



*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.

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

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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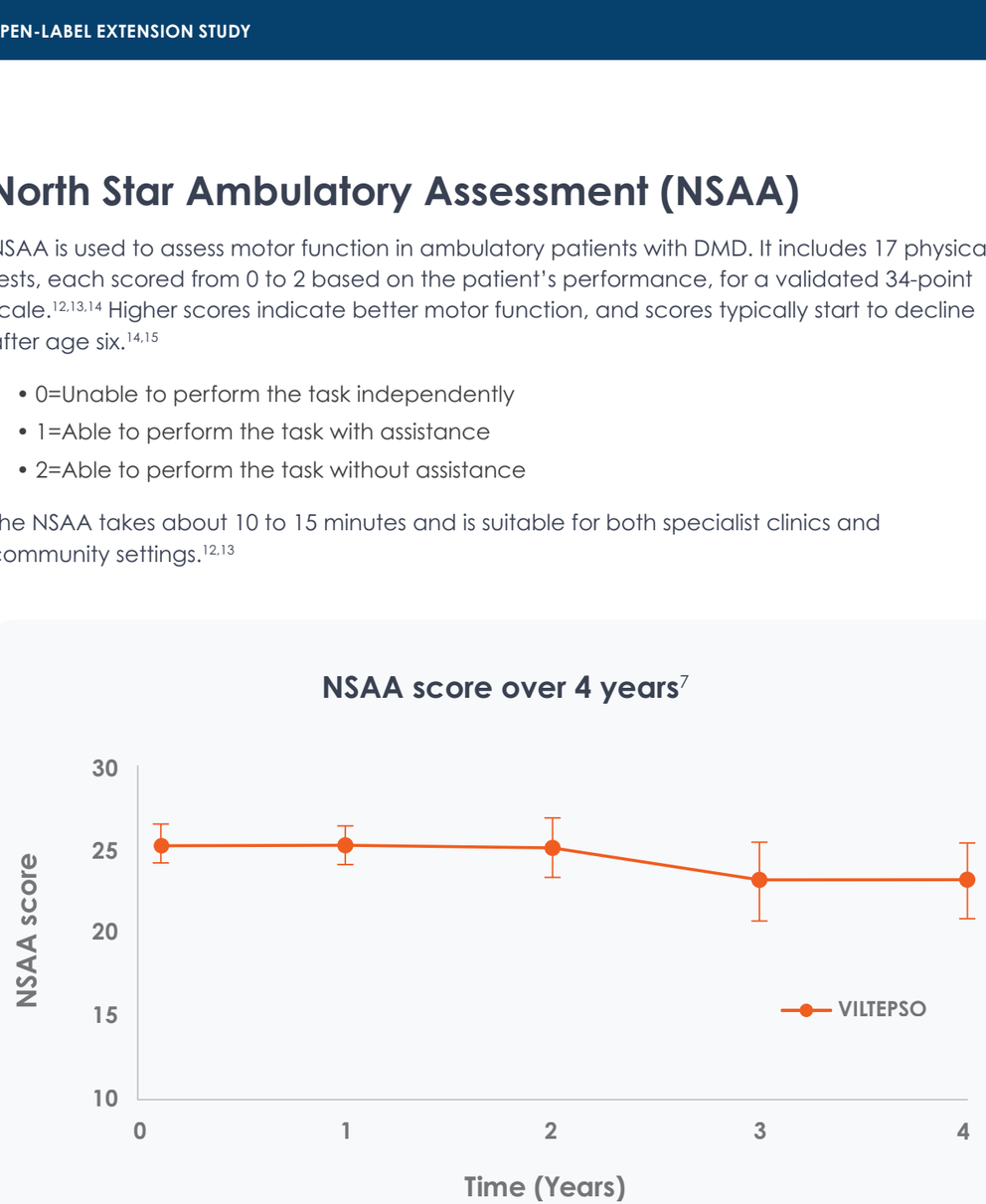
North Star Ambulatory Assessment (NSAA)

NSAA is used to assess motor function in ambulatory patients with DMD. It includes 17 physical tests, each scored from 0 to 2 based on the patient's performance, for a validated 34-point scale.[12,13,14] Higher scores indicate better motor function, and scores typically start to decline after age six.[14,15]

- 0=Unable to perform the task independently
- 1=Able to perform the task with assistance
- 2=Able to perform the task without assistance

The NSAA takes about 10 to 15 minutes and is suitable for both specialist clinics and community settings.[12,13]

NSAA score over 4 years[7]



NSAA was added late to the Duchenne natural history study protocol; the comparator group did not have sufficient data on NSAA for comparison. Functional data are not in the US Prescribing information.

Safety assessment for 4-year, open-label extension study[7]

	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

Three patients had SAEs, none of which were related to the study drug.
AE=adverse event; TEAE=treatment-emergent AE; SAE=serious AE; wk=week.

Side effects with VILTEPSO were mild or moderate, with no treatment-related adverse events.[7]

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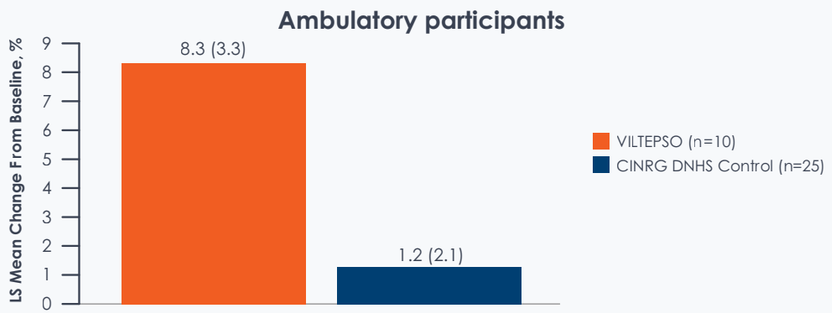
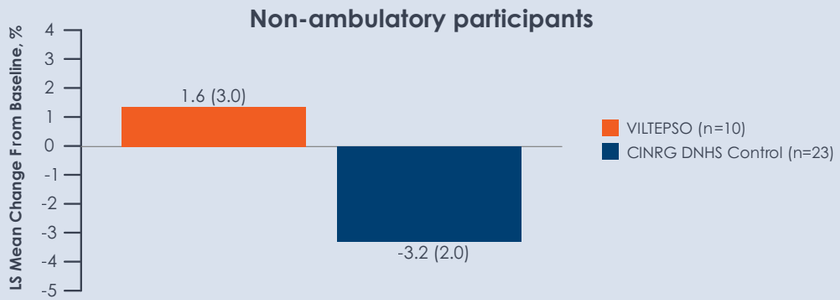
Galactic53 phase 2 study data[8]

Galactic53 was a phase 2, open-label, multicenter study in ambulatory and non-ambulatory males ages 8 years and older with a confirmed DMD mutation amenable to exon 53 skipping. Participants received a weekly infusion of VILTEPSO (80 mg/kg) for 48 weeks.[8]

Pulmonary function was evaluated using forced vital capacity[8]

Change from baseline in FVC%p at week 49[8]





Percent predicted forced vital capacity (FVC%p) is the ratio of an individual's measured FVC to the average FVC among those with the same gender, height, and weight.[8]

Data presented as LS mean (standard error).

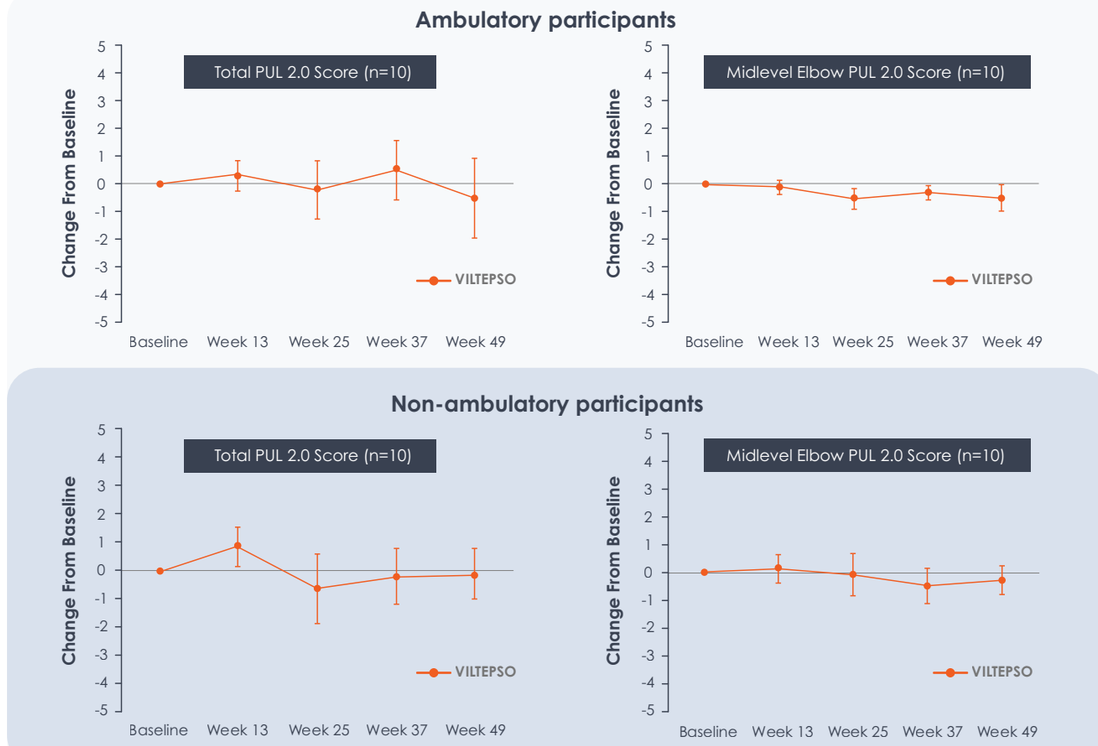
Control subjects were compared with an external control cohort group-matched for multiple variables.

CINRG=Cooperative International Neuromuscular Research Group; DNHS=Duchenne Natural History Study; FVC%p=percent predicted forced vital capacity; LS=least squares.

Pulmonary tests were compared to Duchenne natural history data as the control group rather than to placebo. Galactic53 study data are not included in the US Prescribing Information.

Motor function was measured using total and midlevel elbow scores from performance of upper limb (PUL 2.0)[8]

PUL 2.0 is a validated 42-point scale to evaluate upper limb functionality in individuals with DMD.[16]



Data presented as LS mean (standard error). LS=least squares; PUL=Performance of Upper Limb.

Data was not collected for the natural history control cohort. Galactic53 study data are not included in the US Prescribing Information.

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.

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Primary endpoint: Safety assessment for 48-week Galactic53 study[8]

Participants with:	VILTEPSO 80 mg/kg/wk N=20*
Any TEAE, n (%)	19 (95)
Any treatment-related TEAE[a], n (%)	4 (20)
Discontinuation due to TEAE, n (%)	0
Any serious treatment-related AE, n (%)	0
Death, n (%)	0

[a]Hematuria (n=2), allergic reaction (n=1), and hypertension (n=1).

In the safety population, 95% (19/20) of participants reported TEAEs; all TEAEs were mild or moderate in severity. No participants discontinued VILTEPSO due to TEAEs, and no serious adverse events or deaths occurred during the study.[8]

TEAEs in >1 participant	VILTEPSO 80 mg/kg/wk N=20*
COVID-19 infection	6 (30)
Headache	4 (20)
Hematuria	4 (20)
Nasopharyngitis	3 (15)
Upper respiratory tract infection	3 (15)
Diarrhea	2 (10)
Food poisoning	2 (10)
Influenza	2 (10)
Joint injury	2 (10)
Pain in extremity	2 (10)
Pyrexia	2 (10)
Rhinitis	2 (10)

*Includes both ambulatory and non-ambulatory participants. TEAE = treatment-emergent adverse event

In a clinical trial, VILTEPSO was proven to increase dystrophin in 100% of exon 53 skipping amenable DMD patients.[1]

At week 25, mean increase in dystrophin expression was nearly 6% of normal with VILTEPSO (80 mg/kg/wk) vs 0.6% at baseline (n=8).[1]

Find more efficacy and safety data on VILTEPSO.com/hcp/efficacy-and-safety

References: 1. Viltepso [prescribing information]. Paramus, NJ: NS Pharma, Inc.; 2021. **2.** Crozier RA, Magnus L, Wood M, Previtera ML. Four-year patient experience on viltolarsen. Presented at: Muscular Dystrophy Association Annual Meeting; 2025 March 16-19; Dallas, TX. **3.** Data on file, NS Pharma, Inc. **4.** Echevarría L, Aupy P, Goyenvalle A. Exon-skipping advances for Duchenne muscular dystrophy. *Hum Mol Genet.* 2018;27(R2):R163-R172. doi:10.1093/hmg/ddy171. **5.** Nakamura A. Mutation-based therapeutic strategies for Duchenne muscular dystrophy: From genetic diagnosis to therapy. *J Pers Med.* 2019;9(1):16. Published 2019 Mar 4. doi:10.3390/jpm9010016. **6.** Alberts B, Johnson A, Lewis J, et al. *Molecular Biology of the Cell.* 4th ed. New York: Garland Science; 2002. From RNA to Protein. Available from: https://www.ncbi.nlm.nih.gov/books/NBK26829/. **7.** Clemens PR, Rao VK, Connolly AM, et al. Efficacy and safety of viltolarsen in boys with Duchenne muscular dystrophy: Results from the phase 2, open-label, 4-year extension study. *J Neuromuscul Dis.* 2023;10(3):439-447. doi:10.3233/JND-221656. **8.** Harper, AD, Topaloglu, H, Mercuri E, et al. Safety and efficacy of viltolarsen in ambulatory and nonambulatory males with Duchenne muscular dystrophy. *Sci Rep.* 2024;14:23488. doi:10.1038/s41598-024-70783-y. **9.** Clinicaltrials.gov. Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys With DMD (RACER53). Updated October 27, 2023. Accessed December 2024. Available from: https://clinicaltrials.gov/study/ NCT04060199. **10.** Clemens PR, Rao VK, Connolly AM, et al; for the CINRG DNHS Investigators. Safety, tolerability, and efficacy of viltolarsen in boys with Duchenne muscular dystrophy amenable to exon 53 skipping: a phase 2 randomized clinical trial. *JAMA Neurol.* 2020;77(8):982-991. doi:10.1001/jamaneurol.2020.1264. **11.** US National Library of Medicine. Extension study of NS- 065/NCNP-01 in boys with Duchenne muscular dystrophy (DMD). ClinicalTrials.gov Identifier: NCT03167255. **12.** Scott E, Eagle M, Mayhew A, et al. Development of a functional assessment scale for ambulatory boys with Duchenne muscular dystrophy. *Physiother Res Int.* 2012;17(2):101-109. doi:10.1002/pri.520. **13.** Mazzone ES, Messina S, Vasco G, et al. Reliability of the North Star Ambulatory Assessment in a multicentric setting. *Neuromuscul Disord.* 2009;19(7):458-461. doi:10.1016/j.nmd.2009.06.368. **14.** Muntoni F, Domingos J, Manzur AY, et al. Categorising trajectories and individual item changes of the North Star Ambulatory Assessment in patients with Duchenne muscular dystrophy. *PLoS One.* 2019;14(9):e0221097. doi:10.1371/journal.pone.0221097. **15.** Mayhew AG, Cano SJ, Scott E, et al. Detecting meaningful change using the North Star Ambulatory Assessment in Duchenne muscular dystrophy. *Dev Med Child Neurol.* 2013;55(11):1046-1052. doi:10.1111/dmcn.12220. **16.** Pane M, Coratti G, Bovis F, et al. Longitudinal analysis of PUL 2.0 domains in ambulant and non-ambulant Duchenne muscular dystrophy patients: How do they change in relation to functional ability? *J Neuromuscul Dis.* 2023;77(10):567-57.

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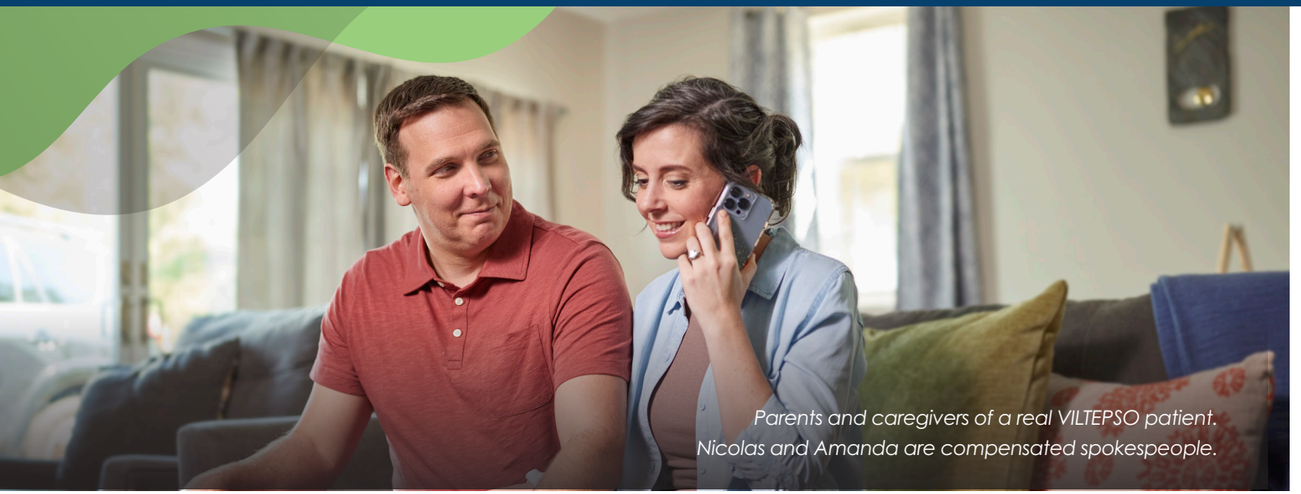


Parents and caregivers of a real VILTEPSO patient.
Nicolas and Amanda are compensated spokespeople.

NS Support®
NS PHARMA **ACCESS SOLUTIONS**

NS Support is available for assistance

Our experienced team is dedicated to assisting patients, caregivers, and healthcare professionals throughout the treatment journey.

FOR PATIENTS AND CAREGIVERS

- Individualized support and resources
- Help with understanding insurance coverage
- 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
- Helpful Patient Authorization Form and Co-pay Program Reimbursement Form

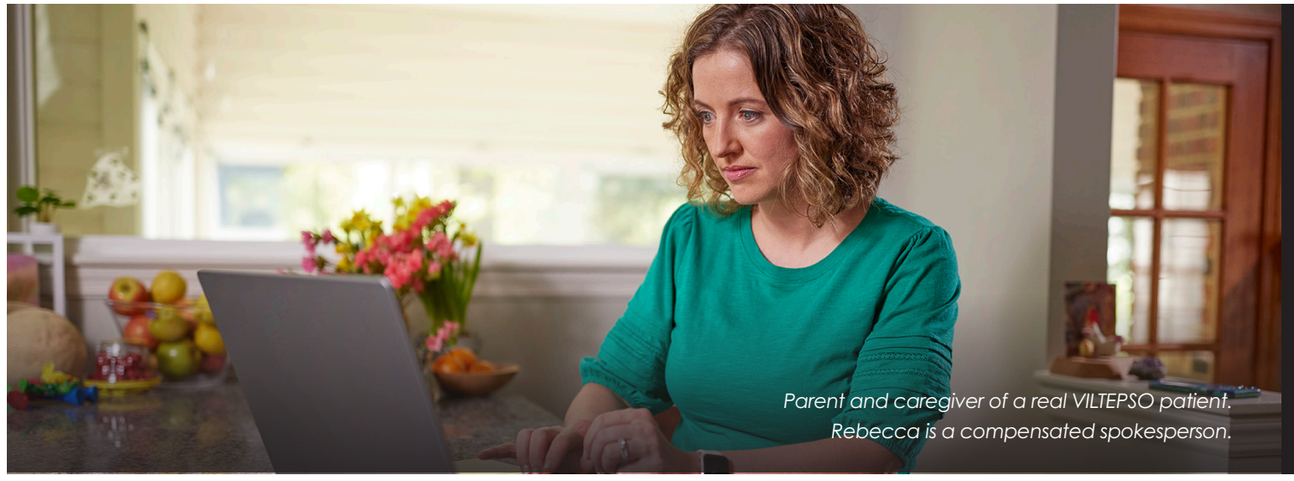
*Patient must not be a participant in a federal or state-funded healthcare program including but not limited to Medicare, Medicaid, Indian Health Service, Department of Defense, or any other federal or state government assistance program. Patient must be a citizen or a permanent resident of the US or its territories and reside in the US or its territories where co-pay assistance is not prohibited.

FOR HEALTHCARE PROFESSIONALS

- Patient Start Form to enroll patients in NS Support
- Product Order Form for streamlined product acquisition options
- Online tool to generate a Letter of Medical Necessity (LMN) for VILTEPSO, whether a patient needs to get prior authorization, get reauthorization, or appeal a denial
- Rapid benefits investigation and verification
- Highly responsive reimbursement support and follow-up calls
- Insights about infusion site options for patients

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

Monday–Friday, 8 AM–8 PM ET



Parent and caregiver of a real VILTEPSO patient.
Rebecca is a compensated spokesperson.

Access helpful tools and forms on VILTEPSO.com/support

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