

REAL-WORLD PATIENT AND TREATMENT CHARACTERISTICS IN CHILDREN UNDER 4 YEARS OLD WITH DUCHENNE MUSCULAR DYSTROPHY RECEIVING THE EXON-SKIPPING THERAPY, VILTOLARSEN

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INTRODUCTION

- Duchenne muscular dystrophy (DMD) is an X-linked disorder caused by mutations in the dystrophin gene, leading to progressive muscle degeneration and premature death¹
- Early diagnosis and intervention in DMD may preserve muscle function and improve long-term outcomes¹
- In December 2025, the US Department of Health and Human Services added DMD to the Recommended Uniform Screening Panel (RUSP) for detection in newborns²
- Exon-skipping therapies can restore the open-reading frame of dystrophin pre-mRNA, producing a shortened dystrophin protein that contains essential functional domains³
- Viltolarsen is a commercially available therapy for DMD in patients with mutations amenable to exon 53 skipping,⁴ representing ~8% of the DMD population⁵
- While clinical trials have demonstrated the efficacy and safety of viltolarsen over 4 years of treatment, enrollment in these studies was restricted to patients aged ≥4 years⁶⁻⁹
- The real-world use of viltolarsen in patients <4 years remains understudied, despite no age restrictions on the therapy.⁴ The recent addition of DMD to the RUSP creates an urgent need for these data to guide earlier intervention

OBJECTIVE

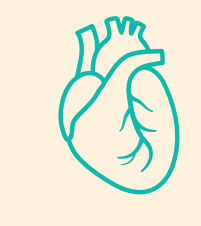
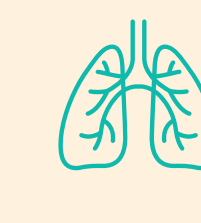
- To assess real-world clinical characteristics and treatment patterns in male children <4 years of age with DMD prior to initiating viltolarsen in the US

METHODS

- A retrospective, observational cohort study was conducted using US claims data from Veeva Compass Patient Database (Anonymous Patient Longitudinal Data)
- Male patients were included if they met all the following: (1) had ≥1 diagnosis claim for DMD (International Classification of Diseases, Tenth Revision, Clinical Modification [ICD-10-CM] diagnosis code G71.01) between January 1, 2017, and October 14, 2025; (2) had ≥2 claims for viltolarsen at least 5 days apart between September 1, 2020, and October 14, 2025; and (3) were aged <4 years at their first viltolarsen claim (index date). Age on claim date was calculated using December 31 as the date of birth for each patient. No minimum continuous enrollment prior to the index date was required. Data with incorrect birth information were excluded from this analysis (n=1)
- Analyses included the presence of cardiac, chronic respiratory, neurological, and orthopedic conditions; prior treatments (exon-skipping therapy, gene therapy, corticosteroids, and cardiac medications); and the use of central venous access devices (CVADs), assisted ventilation, and cough-stimulating devices, using all available data prior to and including the index date (i.e., prior to viltolarsen initiation)

RESULTS

- Of the 115 male patients with claims for viltolarsen, 12 were aged <4 years at their first viltolarsen infusion and met the inclusion criteria
- Patients represented an early-DMD population, with a mean (standard deviation) age of 2.1 (1.1) years and a median (range) age of 2.5 (0.0-3.0) years at viltolarsen initiation

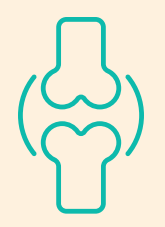
Table 1. Cardiac and chronic respiratory conditions prior to viltolarsen initiation*		N=12
	Any cardiac condition pre-index, n (%)**†	3 (25)
	Cardiac arrhythmia	3 (25)
	Cardiomyopathy	1 (8)
	Cardiac conduction disorder	0 (0)
	Cardiac device or transplant	0 (0)
	Heart failure	0 (0)
	Ischemic heart disease	0 (0)
	Valvular heart disease	0 (0)
	Any chronic respiratory condition pre-index, n (%)**‡	2 (17)
*Non-mutually exclusive groups. †Defined as presence of ≥1 claim related to any of the listed cardiac conditions in this table. Individual patients may have had more than one claim for different pre-index conditions. ‡Defined as presence of ≥1 claim for chronic upper respiratory conditions (e.g., rhinitis), chronic obstructive pulmonary disease (including bronchitis, emphysema), asthma, bronchiectasis, interstitial lung diseases (e.g., pulmonary fibrosis, pneumoconiosis), cystic fibrosis, respiratory failure/insufficiency, or chronic lung allograft dysfunction.		

- Three (25%) of the patients had claims suggestive of a cardiac condition prior to viltolarsen initiation, with claims related to cardiac arrhythmia and cardiomyopathy being recorded (**Table 1**)
- Two (17%) patients had claims suggestive of chronic respiratory conditions

References

1. Birkkrant DJ, et al. *Lancet Neurol.* 2018;17(3):251-67. 2. Federal Register. Addition of Duchenne Muscular Dystrophy to the Recommended Uniform Screening Panel. Available at: <https://www.federalregister.gov/documents/2025/12/22/2025-23573/addition-of-duchenne-muscular-dystrophy-to-the-recommended-uniform-screening-panel> (Accessed August 25, 2026). 3. Roshmi RR and Yokota T. *Clin Pharmacol.* 2021;13:235-42. 4. VILTEPSO (viltolarsen) injection, for intravenous use. Prescribing information. NS Pharma, Inc.; 2021. 5. Bladen CL, et al. *Hum Mutat.* 2015;36(4):395-402. 6. Clemens PR, et al. *J Neuromuscul Dis.* 2023;10(3):439-47. 7. Clemens PR, et al. *JAMA Neurol.* 2020;77(8):982-91. 8. Komaki H, et al. *Ann Clin Transl Neurol.* 2020;7(12):2393-408. 9. Harper AD, et al. *Sci Rep.* 2024;14(1):23488.

Table 2. Neurological and orthopedic conditions prior to viltolarsen initiation

		N=12
	Any neurological condition pre-index, n (%)*	9 (75)
	Neurodevelopmental	9 (75)
	Sleep disorder	4 (33)
	Seizure-related†	2 (17)
	Any orthopedic condition pre-index, n (%)*	9 (75)
	Other deformities, including foot	4 (33)
	Joint/non-fracture	3 (25)
	Spinal deformity	3 (25)
	Orthopedic pain	2 (17)
	Fractures	1 (8)
	Orthopedic contracture	0 (0)

*Non-mutually exclusive groups.
†Defined as presence of ≥1 claim for epilepsy and recurrent seizures, febrile convulsion, functional conversion disorder with seizure, or convulsions of newborns.

- Most patients (75%) had claims indicating neurodevelopmental issues (**Table 2**)
- Claims suggestive of orthopedic comorbidities were recorded in the majority of patients (75%), with other deformities (including foot), joint/non-fracture, and spinal deformity, being most common, followed by orthopedic pain (**Table 2**)

Table 3. Medication and CVAD usage prior to viltolarsen initiation

		N=12
	Other DMD exon-skipping medications, n (%)**†	1 (8)
	DMD gene therapy, n (%)**‡	0 (0)
	Any corticosteroid, n (%)**§	1 (8)
	DMD-approved corticosteroids, n (%)**§	0 (0)
	Any cardiac medication, n (%)**¶	0 (0)
	Any CVAD procedure, n (%)*	4 (33)

*Non-mutually exclusive groups.
†Other DMD exon-skipping medications' includes eteplirsen and golodirsen.
‡DMD gene therapy' includes delandistrogene moxeparvovec.
§Any corticosteroid' includes deflazacort, methylprednisolone, prednisolone, prednisone, and vamorolone, while 'DMD-approved corticosteroids' includes deflazacort and vamorolone.
¶Any cardiac medication' includes angiotensin-converting enzyme inhibitors, angiotensin receptor blockers, angiotensin receptor neprilysin inhibitors, calcium channel blockers, diuretics, oral beta-blockers, mineralocorticoid receptor antagonists, and sodium-glucose cotransporter-2 inhibitors.
CVAD, central venous access device; DMD, Duchenne muscular dystrophy.

- One patient had previously received exon-skipping therapy, indicating that viltolarsen was the first exon-skipping therapy for most patients (**Table 3**)
- No patients had previously received gene therapy prior to initiating viltolarsen
- Corticosteroid use was uncommon before patients started viltolarsen. One patient had a claim for a non-DMD-approved oral corticosteroid; use was non-continuous (defined as <6 months use or with gaps ≥14 days), suggesting treatment for an acute, non-DMD condition
- A third of the patients had claims for CVAD procedures

Assisted ventilation or cough-stimulating device

- One (8%) patient had claims suggestive of non-invasive assisted ventilation
- No patients had claims suggestive of using a cough-assist device

LIMITATIONS

- Findings are limited by a small sample size (n = 12) and reliance upon a mixture of open and closed claims data from a variety of sources and are subject to coding errors and may lack clinical granularity (e.g., specific mutations, functional assessments)
- Results may not fully reflect broader DMD populations, as the database is limited to US participants and excludes uninsured and exclusive Medicaid patients
- Date-of-birth approximation may have resulted in minor age misclassifications
- Finally, the absence of a minimum baseline look-back period introduces ascertainment bias; patients with longer observation histories or greater disease severity likely have more complete capture of prior conditions, treatments, and equipment use

CONCLUSIONS

- We report clinical characteristics and treatment patterns in children with DMD aged <4 years receiving viltolarsen in real-world clinical practice, a population not represented in clinical trials
- Despite their young age, claims data suggest that many patients had early neurodevelopmental symptoms
- Viltolarsen was the first exon-skipping therapy for most patients
- A third of patients had ports, reflecting an option for viltolarsen delivery in very young children
- With DMD added to the RUSP, this research provides valuable real-world insights into early initiation of viltolarsen in a young cohort of patients with DMD, helping to inform strategies to optimize early treatment for patients with exon 53 skip-amenable mutations

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Disclosures

All authors report employment at NS Pharma, Inc.