

REAL-WORLD PATIENT AND TREATMENT CHARACTERISTICS IN A NON-AMBULATORY COHORT ≥12 YEARS OLD WITH DUCHENNE MUSCULAR DYSTROPHY RECEIVING VILTOLARSEN, AN EXON-SKIPPING THERAPY

Poster 397

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INTRODUCTION

- Duchenne muscular dystrophy (DMD) is an X-linked muscular disorder caused by dystrophin gene mutations, resulting in progressive muscle loss¹
- Most patients lose ambulation in their second decade, along with declining pulmonary function and increasing reliance on assisted ventilation, and an increased risk of cardiac failure^{1,2}
- Corticosteroid use is recommended to slow muscle degeneration and preserve strength,³ alongside cardiac medications initiated prophylactically before systolic dysfunction or to manage sinus tachycardia⁴
- Exon-skipping therapies can correct deletion mutations by modifying mRNA splicing to restore the open-reading frame, enabling production of a shortened dystrophin protein containing essential functional domains⁵
- Viltolarsen is a commercially available therapy for the treatment of DMD in patients with mutations amenable to exon 53 skipping,⁶ which accounts for ~8% of patients⁷
- The Phase II clinical trial, Galactic53, demonstrated the efficacy and safety of viltolarsen in ambulatory and non-ambulatory males with DMD;⁸ here, real-world data demonstrate that healthcare providers prescribe viltolarsen to older, non-ambulatory patients, including those with comorbidities, often as part of a combination treatment regimen

OBJECTIVE

- To assess real-world clinical characteristics and treatment patterns in non-ambulatory male patients aged ≥12 years 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; (3) were aged ≥12-35 years at their first viltolarsen claim (index date); and (4) had ≥1 claim indicative of non-ambulatory status (a diagnosis or procedure code indicating wheelchair or mobility scooter use, related accessories or maintenance, or bed confinement) any time prior to and including the 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, 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), 37 met the inclusion criteria
- Patients had a mean (standard deviation) age of 17.4 (5.2) years and a median (range) age of 15.0 (12.0-29.0) years at time of viltolarsen initiation (**Figure 1**)
- Mean (standard deviation) age at first claim indicative of non-ambulatory status was 13.0 (4.8) years, while median (range) age was 11.0 (6.0-23.0)

Figure 1. Age at viltolarsen initiation

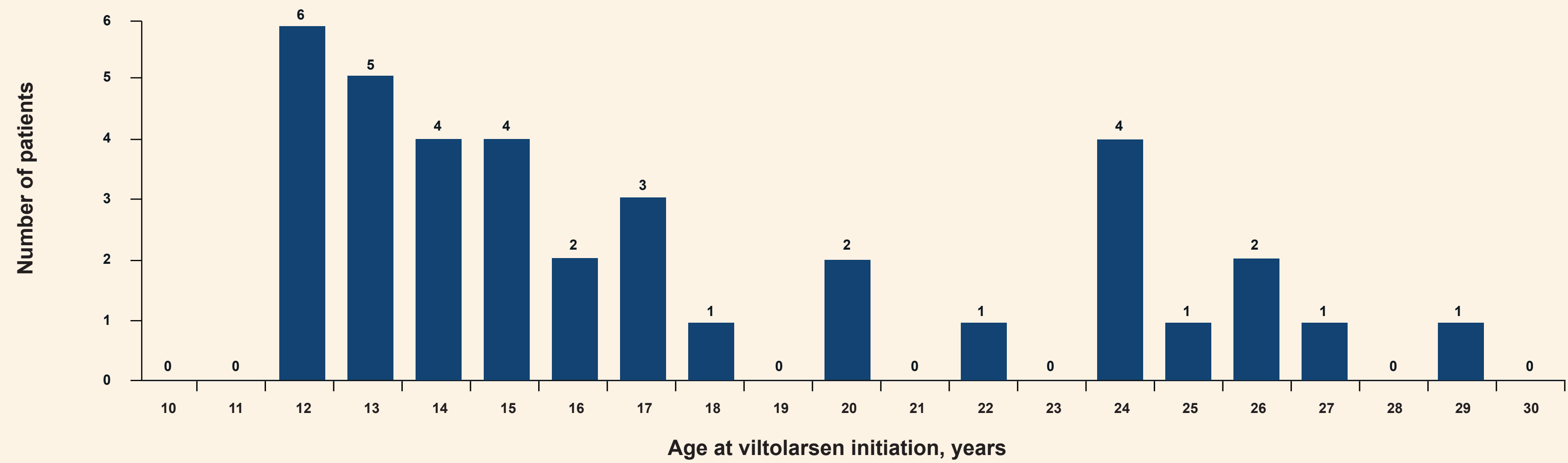
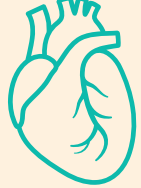
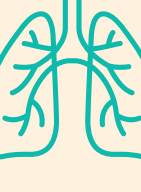


Table 1. Cardiac and chronic respiratory conditions prior to viltolarsen initiation

		N=37
	Any cardiac condition pre-index, n (%) ^{*†}	23 (62)
	Cardiac arrhythmia	14 (38)
	Cardiomyopathy	14 (38)
	Valvular heart disease	7 (19)
	Heart failure	5 (14)
	Cardiac conduction disorder	4 (11)
	Ischemic heart disease	2 (5)
	Cardiac device or transplant	0 (0)
	Any chronic respiratory condition pre-index, n (%) ^{*‡}	16 (43)

^{*}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.

References

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Table 2. Neurological and orthopedic conditions prior to viltolarsen initiation

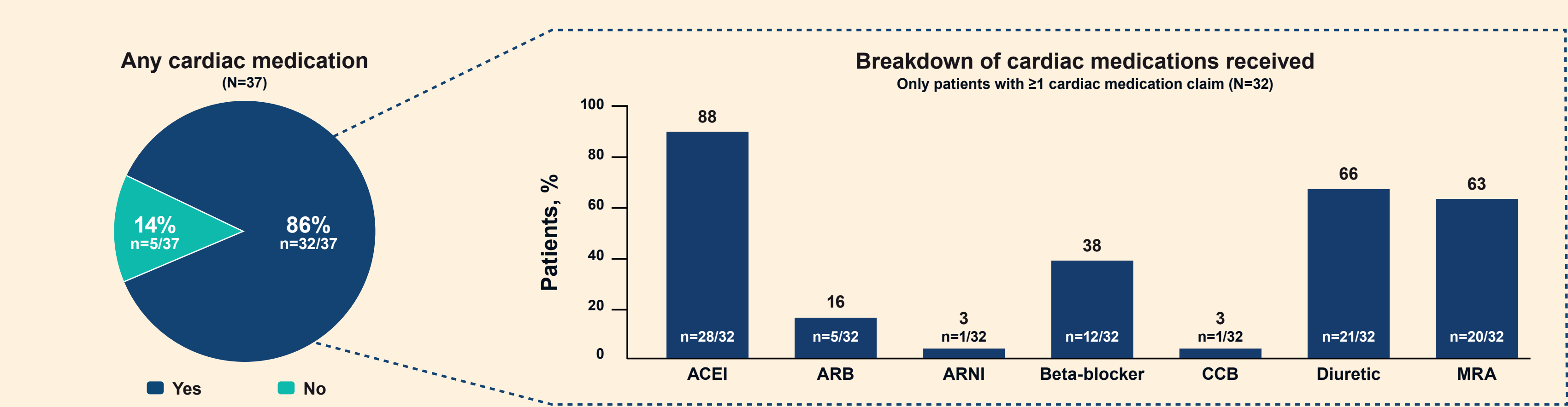
		N=37
	Any neurological condition pre-index, n (%) [*]	26 (70)
	Sleep disorder	23 (62)
	Neurodevelopmental	15 (41)
	Seizure-related [†]	2 (5)
	Any orthopedic condition pre-index, n (%) [*]	36 (97)
	Joint/non-fracture	26 (70)
	Fractures	24 (65)
	Orthopedic pain	24 (65)
	Spinal deformity	23 (62)
	Orthopedic contracture	14 (38)
	Other deformities, including foot	11 (30)

^{*}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.

Medication and CVAD usage prior to viltolarsen initiation

- Six (16%) out of 37 patients received other exon-skipping therapy prior to viltolarsen
- There were no claims for prior DMD gene therapy reported
- Oral corticosteroid use was recorded in 23/37 (62%) patients prior to viltolarsen initiation
 - Of these patients, 11/23 (48%) received a DMD-approved corticosteroid (deflazacort or vamorolone)
 - Continuous oral corticosteroid use (≥6 months use with a <14-day allowable gap) was recorded for 14 (61%) of the 23 patients receiving corticosteroids
- Of the 32/37 (86%) patients who had claims for cardiac medication, most were on an angiotensin-converting enzyme inhibitor (ACEI; 88%), a diuretic (66%), a mineralocorticoid receptor antagonist (MRA; 63%), an oral beta-blocker (38%), and/or an angiotensin receptor blocker (ARB; 16%; **Figure 2**)
- Claims for CVAD procedures were recorded in 9/37 (24%) patients

Figure 2. Cardiac medication usage prior to viltolarsen initiation



No patients received an SGLT-2 inhibitor. Non-mutually exclusive groups.
ACEI, angiotensin-converting enzyme inhibitor; ARB, angiotensin receptor blocker; ARNI, angiotensin receptor neprilysin inhibitor; CCB, calcium channel blocker; MRA, mineralocorticoid receptor antagonist; SGLT-2, sodium-glucose cotransporter-2.

Table 3. Assisted ventilation or cough-stimulating device claims prior to viltolarsen initiation

		N=37
	Any ventilation or cough-stimulating device use pre-index, n (%) [*]	28 (76)
	Invasive assisted ventilation	4 (11)
	Non-invasive assisted ventilation	22 (59)
	Cough-stimulating device	19 (51)

^{*}Non-mutually exclusive groups.

LIMITATIONS

- Findings rely 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

- These real-world data reveal the significant systemic disease burden in non-ambulatory males with DMD aged ≥12-35 years prior to initiating viltolarsen, including high reliance on assisted ventilation and cardiac interventions
- High usage of cardiac medications, in particular ACEIs, diuretics, MRAs, beta-blockers, and ARBs, aligns with guideline recommendations for prophylactic use in patients aged 10 years and older⁴
- Assisted ventilation was widely required, with non-invasive methods being more common than invasive ventilation
- Despite advanced disease, many patients (38%) lacked claims for corticosteroids, suggesting potential sub-optimal treatment of some patients, discontinuation prior to first claim in database, or initiation after viltolarsen
- These real-world findings in older patients with advanced DMD who initiated viltolarsen complement the Galactic53 clinical trial. As the first study to evaluate respiratory outcomes with viltolarsen, Galactic53 demonstrated clinical benefit on pulmonary function and stabilization of upper limb motor function in ambulatory and non-ambulatory patients.⁸ Together these findings highlight the potential to stabilize upper limb and respiratory symptoms, and provide valuable insights to inform clinicians of strategies to optimize treatment approaches for older, non-ambulatory patients with DMD

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Disclosures

All authors report employment at NS Pharma, Inc.