An Open-Claims Data Set Analysis to Evaluate the Persistence of Viltolarsen, an Exon-Skipping Therapy, in Patients With Duchenne Muscular Dystrophy

Poster 57 S

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INTRODUCTION

- Viltolarsen is a Food and Drug Administration (FDA)-approved therapy that became commercially available in August 2020 for the treatment of patients with Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping[1]
- DMD is a severe, X-linked neuromuscular disease caused by the absence of functional dystrophin, most commonly due to deletions in the *DMD* gene that disrupt the dystrophin open-reading frame, leading to progressive muscle degeneration and premature death[2]
- Exon-skipping therapy aims to restore dystrophin production by modifying messenger ribonucleic acid (mRNA) frames to bypass mutated exons, leading to the production of a shortened dystrophin containing essential functional domains[1,3]
- Approximately 8% of patients with DMD have mutations amenable to exon 53 skipping, making them eligible for viltolarsen therapy, which uses antisense oligonucleotides to restore the open-reading frame of the *DMD* gene[1,3]
- Viltolarsen is administered as a weekly infusion in the home or clinic[1], and it is important to maintain once-weekly, uninterrupted treatment[4]
- The clinical efficacy and safety of viltolarsen was demonstrated in an open-label extension to 4 years[5]
- Continued treatment is required to slow disease progression and manage functional decline. The clinical trial setting is necessarily conducive to maintaining persistence, but there is a lack of real-world long-term data to inform an understanding of viltolarsen in practice[4]

OBJECTIVE

- To report estimated overall treatment persistence for patients treated with viltolarsen over 1-, 2-, and 3-year periods based on a US open-claims dataset

METHODS

- We conducted a longitudinal, non-interventional, retrospective cohort data analysis of anonymized claims from male patients (≤30 years of age) with DMD obtained from Veeva Compass Patient (longitudinal anonymous patient-level data) to estimate the probability of persistence to viltolarsen
- DMD diagnosis was confirmed by the International Classification of Diseases, Tenth Revision, Clinical Modification (ICD-10-CM) diagnosis code G71.01 (Duchenne or Becker muscular dystrophy) plus receipt of viltolarsen claims for a minimum of two distinct calendar dates
- Patients were divided into three groups, based on their first viltolarsen claim date **(Figure 1)**
- Persistence was defined as time from first prescription fill to one of the following: failure, loss to follow-up, or end of study period. A maximum gap of 61 days was allowed between claims

Figure 1. Study design

*Clinical Modification (ICD-10-CM) diagnosis code G71.01 (Duchenne or Becker muscular dystrophy);
†Receipt of viltolarsen claims for a minimum of two distinct calendar dates.
DMD, Duchenne muscular dystrophy.

RESULTS

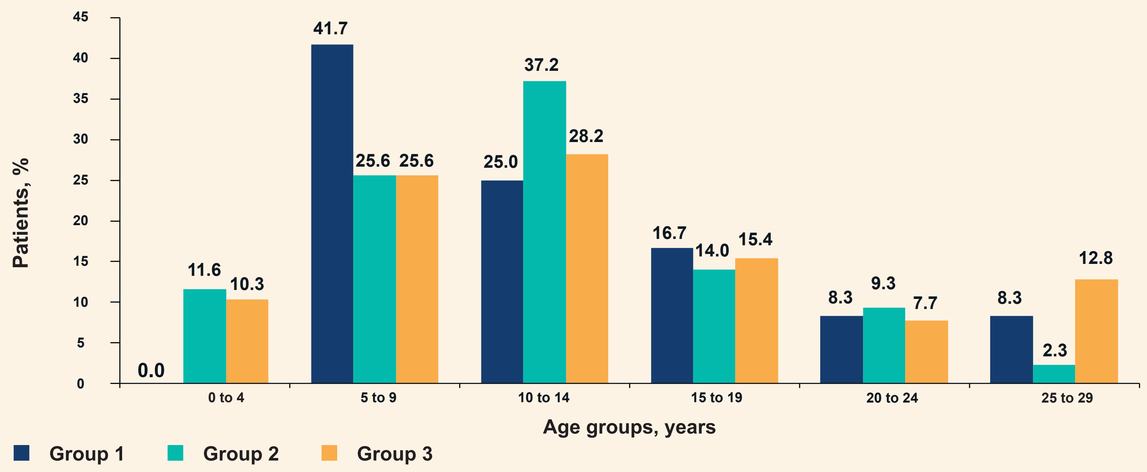
- Ninety-four patients were identified; of these, five patients failed between the first and second claim and were therefore not included in the main analysis
- The patients represented all regions of the US (**Table 1**). The median (interquartile range [IQR]) age of patients included in the sample was 12.5 (9.3, 16.8) years, and the distribution of patient ages was generally consistent across the three groups (**Table 1; Figure 2**)

Table 1. Patient characteristics

	Group 1	Group 2	Group 3	Overall
N (%)	12 (12.8)	43 (45.7)	39 (41.5)	94 (100)
Age				
Mean, years (SD)	14.1 (6.4)	12.9 (5.5)	13.6 (7.3)	13.4 (6.4)
Median, years	13.6	12.3	12.5	12.5
(IQR)	(8.7, 18.8)	(10.1, 16.0)	(8.0, 19.0)	(9.3, 16.8)
Region, n (%)				
Northeastern	3 (25.0)	5 (11.6)	6 (15.4)	14 (14.9)
South	5 (41.7)	15 (34.9)	14 (35.9)	34 (36.2)
Midwest	2 (16.7)	14 (32.6)	6 (15.4)	22 (23.4)
West	2 (16.7)	9 (20.9)	13 (33.3)	24 (25.5)

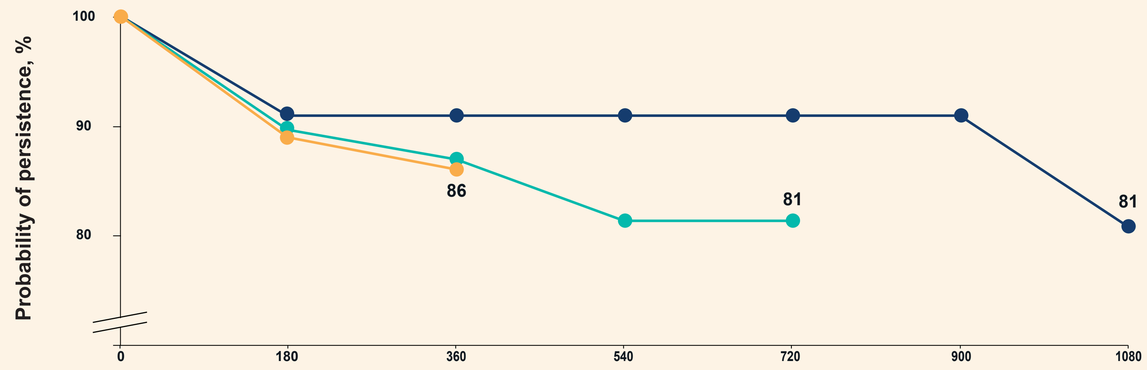
IQR, interquartile range; SD, standard deviation.

Figure 2. Patient age at baseline



- Within the respective cohorts, the probability of persistence with viltolarsen treatment was 81% over 3 years in Group 1 (n=12), 81% over 2 years in Group 2 (n=41), and 86% over 1 year in Group 3 (n=36) (**Figure 3**)

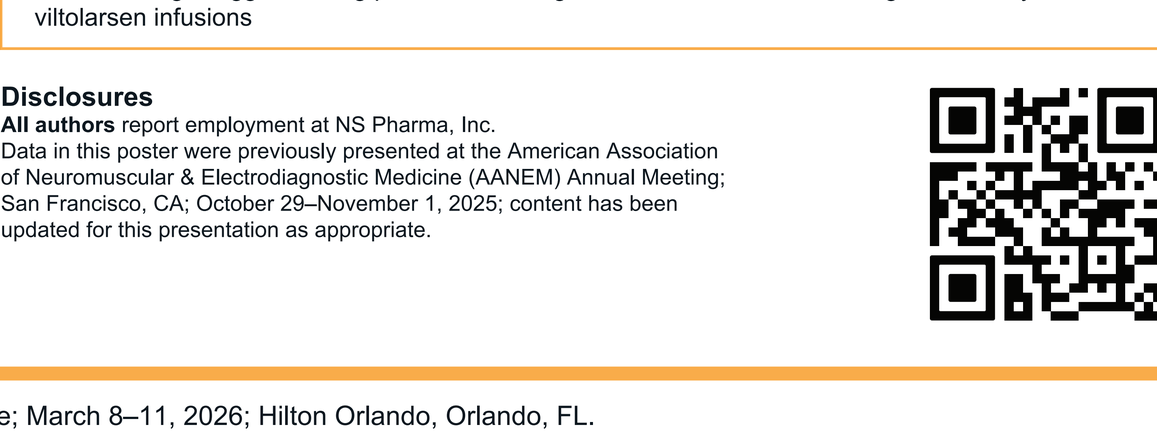
Figure 3. Probability of persistence with time*



*Five patients failed between the first and second claim and are therefore not included in the main analysis.

- Sensitivity analysis was conducted, which considered other non-exon-skipping claims following a last observed viltolarsen dose to determine which patients are more likely to have discontinued rather than being "lost to follow-up"
- When these patients were accounted for, the probability of persistence with viltolarsen treatment was 67% over 3 years in Group 1 (n=12), 68% over 2 years in Group 2 (n=41), and 83% over 1 year in Group 3 (n=36) (**Figure 4**)

Figure 4. Probability of persistence with time (sensitivity analysis)



CONCLUSIONS

- We report high real-world persistence rates with viltolarsen sustained through to 3 years, supporting its long-term viability and potential for durable therapeutic benefit in clinical practice[3,5]
- The sensitivity analysis used stricter definitions of "failure" and therefore provides more conservative estimates of persistence than the main analysis
- Our analysis addresses gaps in prior research through examination of patient persistence to viltolarsen across three mutually distinct patient subgroups with opportunity for up to 1-, 2-, or 3-years' exposure to viltolarsen exon-skipping therapy, respectively
- Our analysis is limited by the exclusion of patients who restarted viltolarsen treatment after an allowable gap in analysis and the inherent constraints of using open-claims data
- These findings suggest strong patient and caregiver commitment to continuing with weekly viltolarsen infusions

References

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Disclosures

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