

Persistence of Viltolarsen, an Exon-Skipping Therapy, in Patients With Duchenne Muscular Dystrophy Using an Open-Claims Data Set

Poster 294

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INTRODUCTION

- Viltolarsen became commercially available in August 2020 for the treatment of patients with Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping¹
- DMD is a severe, X-linked neuromuscular disease caused by the absence of functional dystrophin, leading to progressive muscle degeneration and premature death²
- 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^{1,3}
- Viltolarsen is administered as a weekly infusion in the home or clinic¹, and it is important to maintain once-weekly, uninterrupted treatment⁴
- The clinical efficacy and safety of viltolarsen was demonstrated in an open-label extension to 4 years⁵
- 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 practice⁴

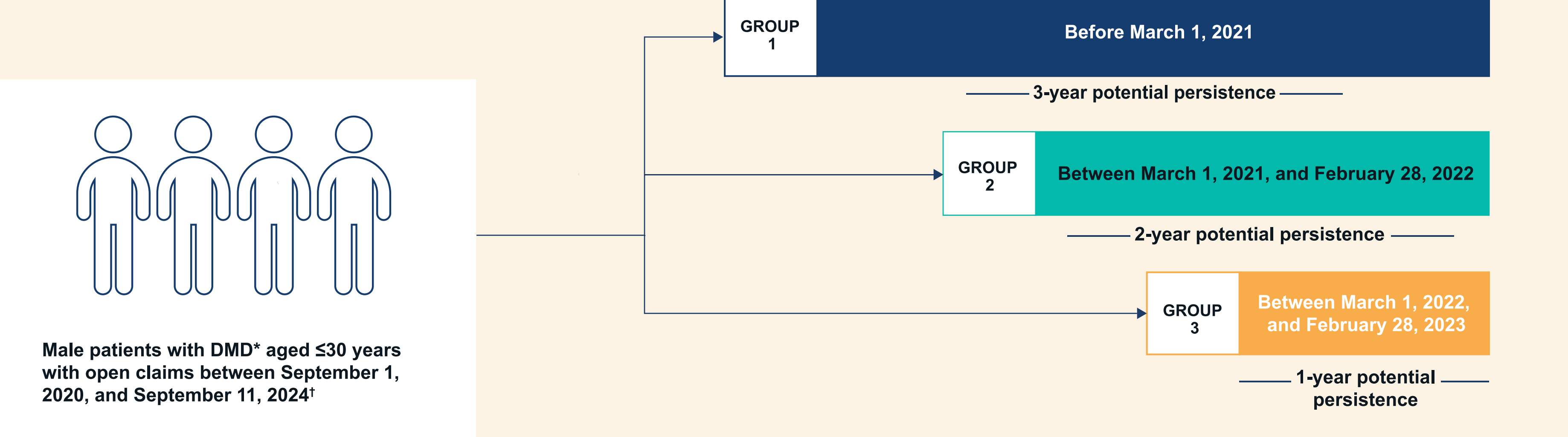
OBJECTIVE

- To report estimated overall treatment persistence for patients treated with viltolarsen over 1-, 2-, and 3-year periods based on an 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**)

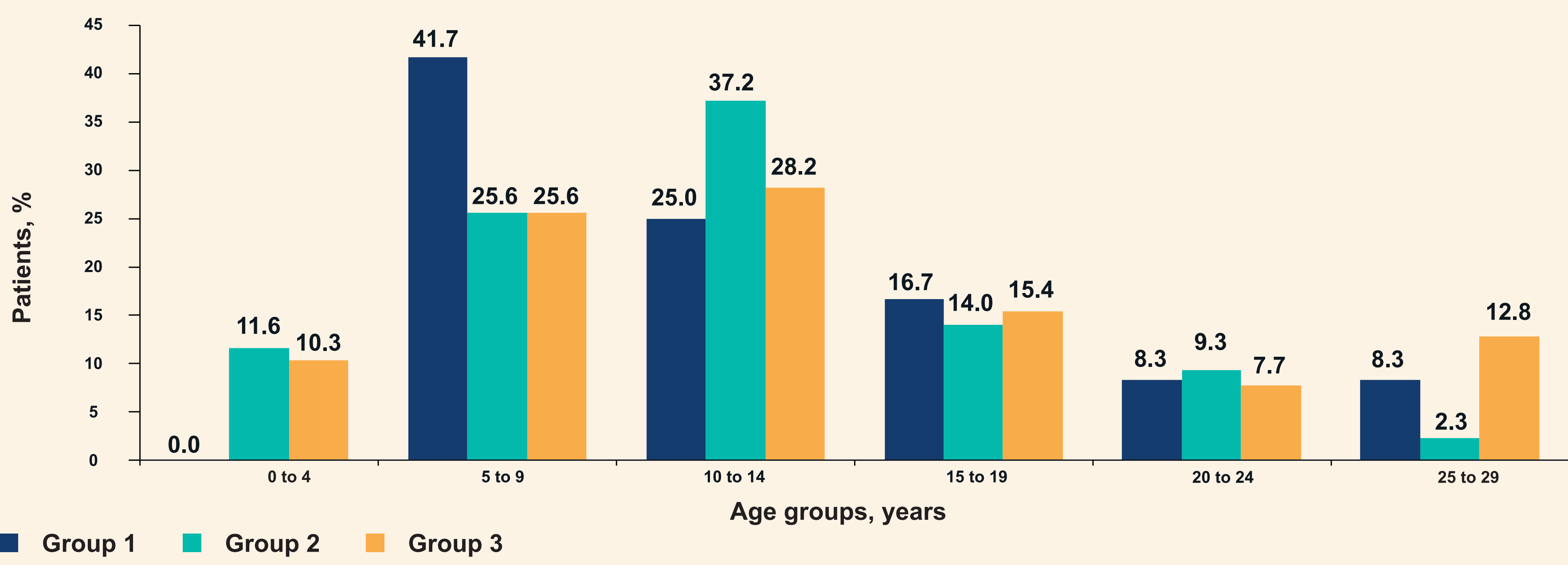
Table 1. Patient characteristics

	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 (IQR)	13.6 (8.7, 18.8)	12.3 (10.1, 16.0)	12.5 (8.0, 19.0)	12.5 (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.

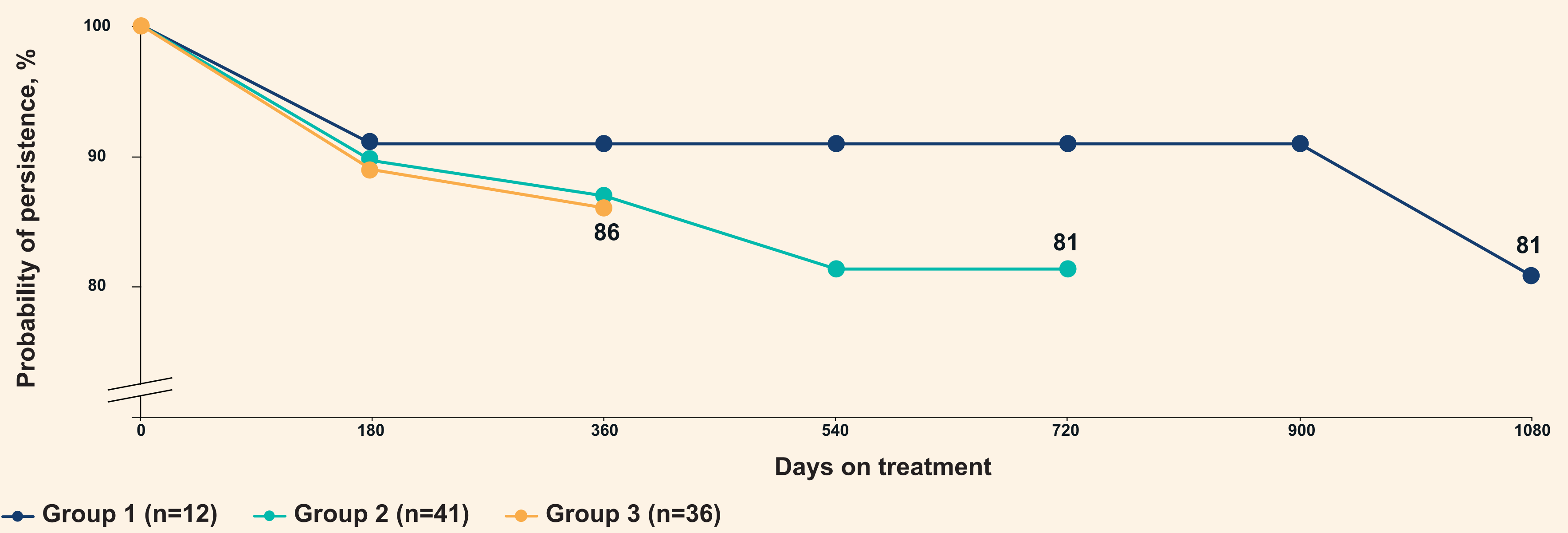
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1. VILTEPSO (viltolarsen) injection, for intravenous use. Prescribing information. NS Pharma, Inc.; 2021.
2. Angulski ABB, et al. *Front Physiol.* 2023;14:1183101.
3. Roshmi RR and Yokota T. *Clin Pharmacol.* 2021;13:235-42.
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Figure 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**)

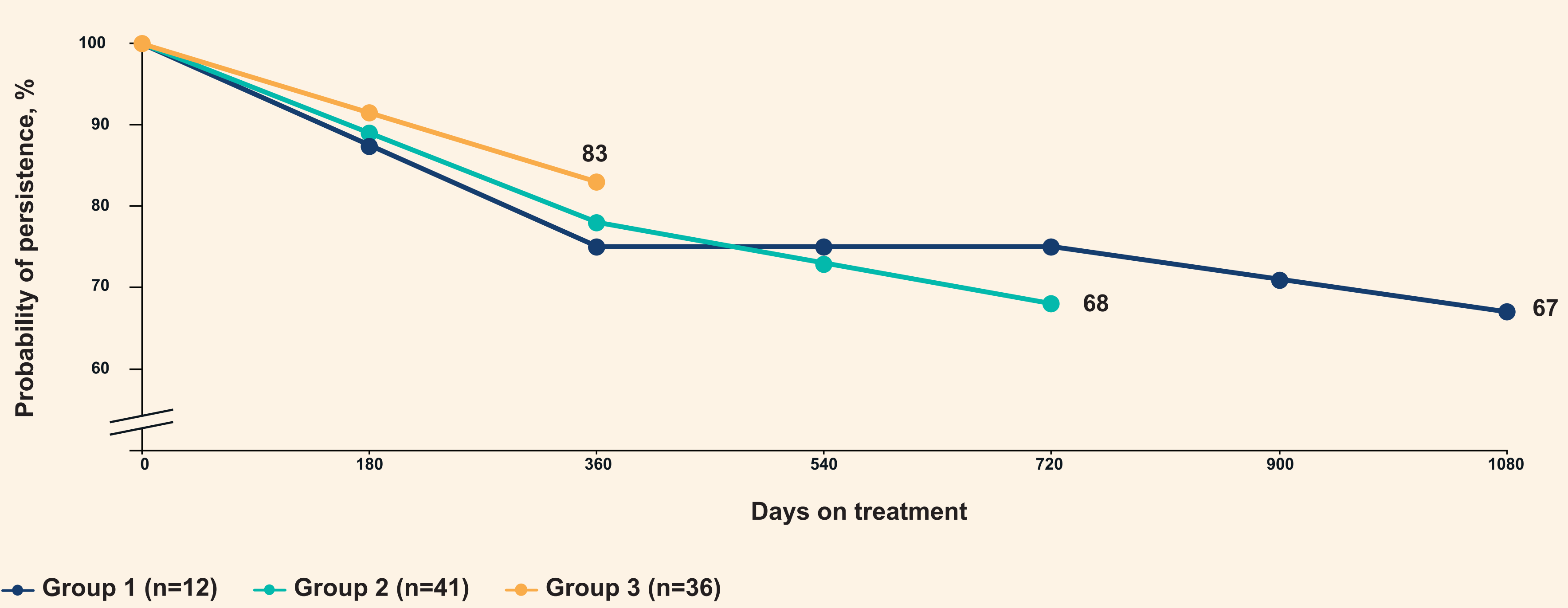
Figure 3. Probability of persistence with time*



*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

Disclosures
All authors report employment at NS Pharma, Inc.

Acknowledgments
Editorial support for this poster was provided by dna Communications and funded by NS Pharma, Inc.