Four-Year Patient Experience on Viltolarsen

Poster 111

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BACKGROUND

- Viltolarsen (VILTEPSO®) is an antisense oligonucleotide that became commercially available in the US in August 2020 and is indicated for the treatment of patients with Duchenne muscular dystrophy (DMD) with a dystrophin gene mutation amenable to exon 53 skipping[1]
- Viltolarsen binds to exon 53 of dystrophin pre-mRNA, resulting in exclusion of that exon and, subsequently, the production of a shortened dystrophin protein containing essential functional portions[1]
- In a previous clinical trial, participants (aged 4 to <10 years at the start of the study) treated with viltolarsen showed significant increases in dystrophin protein levels and stabilized motor function[2]
- In an additional clinical trial, ambulatory and nonambulatory participants (aged ≥8 years) treated with viltolarsen for 48 weeks demonstrated slower decline of pulmonary function compared with matched natural history controls, as well as maintenance of upper limb performance[3]
- Participants in DMD clinical trials must meet certain enrollment criteria, such as age at the start of the study, as well as certain exclusion criteria including limitations on the use of medications that could impact the interpretation of the clinical trial results; unlike real-world patients, clinical trial participants can benefit from receiving a regular and uninterrupted dose of study medication
- With over 4 years since launch, determining and understanding real-world trends in the experience of patients receiving viltolarsen outside of the clinical trial setting is important
- Here, we provide a snapshot of the patient experience on viltolarsen using over 4 years of real-world patient-level data

METHODS

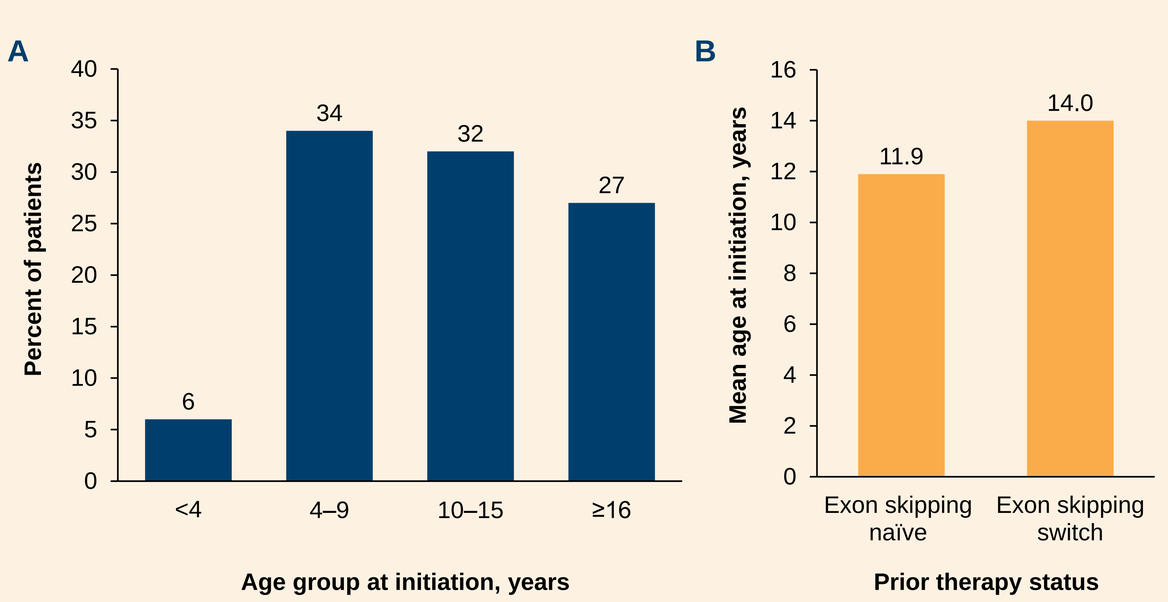
- An internal dataset sourced from specialty pharmacy partners and the viltolarsen patient hub was used for this analysis
- Analysis was performed from launch (August 2020) to November 2024
- Prior therapy information was patient-reported
- Eligible patients must have received ≥1 infusion of viltolarsen
- Compliance or adherence to the prescribed dosing regimen was calculated according to the medication possession ratio (MPR) methodology
  - The MPR measures medication compliance (also referred to as refill adherence) as a percentage; medication compliance is defined as “the act of conforming to the recommendations made by the provider with respect to timing, dosage, and frequency of medication taking”[4]
  - MPR was calculated as days’ supply obtained/dispensing period[5]; early refills can overestimate compliance, and to avoid overstating, compliance for each patient was capped at 100%

RESULTS

Patient age

- The mean age at viltolarsen treatment initiation was 12.4 years (**Figure 1A**)
  - The youngest patient to initiate viltolarsen was 1 year old, whereas the oldest patient was 30 years old
  - Most patients (59%) were ≥10 years old at initiation; 6% were under 4 years of age
- At the time of this analysis, the mean age of patients receiving viltolarsen was 14.4 years
  - At data cutoff, the youngest patient receiving viltolarsen was 1 year old and the oldest was 32 years old
- Patients who received an exon skipping therapy prior to viltolarsen (exon skipping switch) tended to be older at the time of viltolarsen initiation than patients who were naïve to exon skipping therapy (exon skipping naïve; **Figure 1B**)

Figure 1. Distribution of patient age at viltolarsen treatment initiation (A) and mean age at initiation by prior therapy status (B)

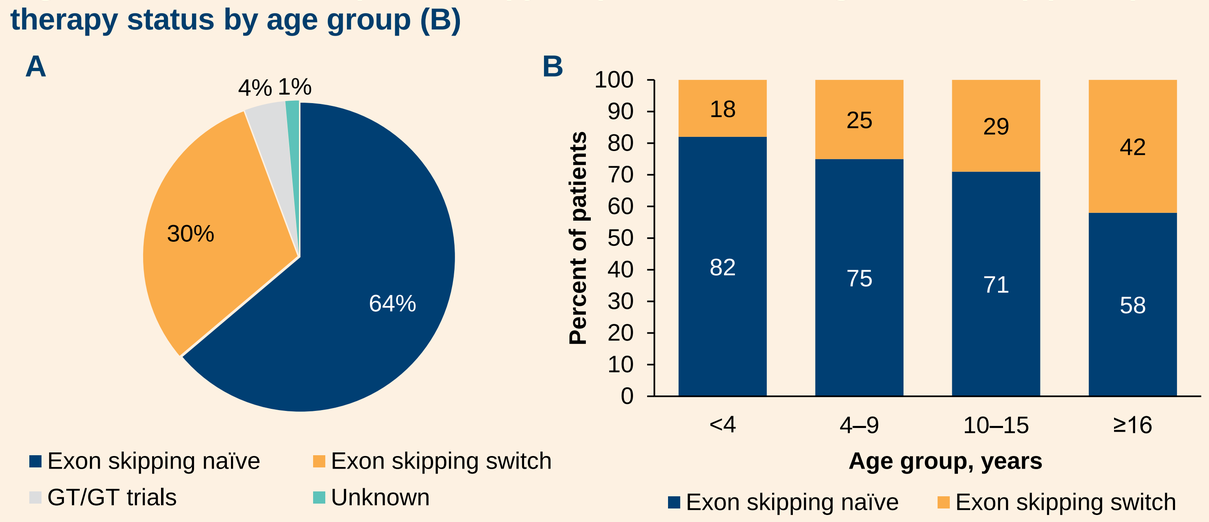


Exon skipping switch denotes patients who received an exon skipping therapy prior to viltolarsen. Prior therapy was patient-reported to the hub. Overall percentage in panel A does not total 100 due to rounding.

Prior exon skipping or gene therapy

- The majority of patients (64%) who received viltolarsen were exon skipping naïve while almost a third of patients (30%) previously received another exon skipping therapy (**Figure 2A**)
- A small proportion of patients (4%) had received gene transfer therapy or were part of a gene transfer therapy clinical trial prior to receiving viltolarsen
- The proportion of patients who received an exon skipping agent prior to viltolarsen were older than those who were exon skipping naïve (**Figure 2B**)

Figure 2. Distribution of prior therapy for patients receiving viltolarsen (A) and prior therapy status by age group (B)

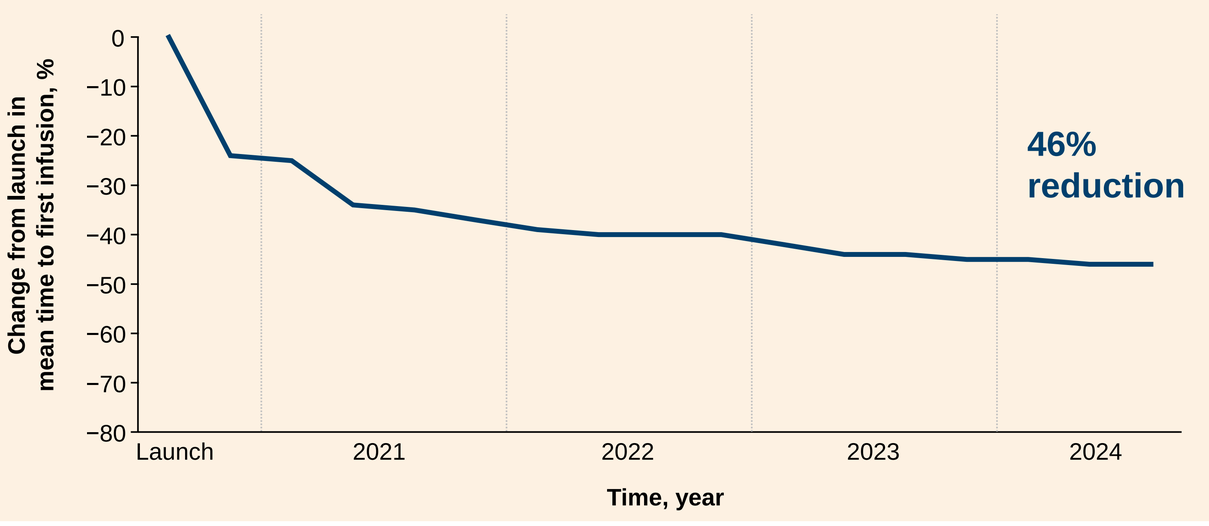


Exon skipping switch denotes patients who received an exon skipping therapy prior to viltolarsen. Prior therapy was patient-reported to the hub. Overall percentage in panel A does not total 100 due to rounding.
GT, gene therapy.

Time to first infusion

- Since the launch of viltolarsen, the mean time from receipt of a prescription to the first infusion decreased by 46% (**Figure 3**)
- The mean time to first infusion differed by age and prior exon skipping or gene therapy
  - Patients younger than 13 years old initiated treatment approximately 32% faster than patients 13 years of age or older
  - Patients who were previously treated with an exon skipping therapy received their first viltolarsen infusion ~30 days faster than those who were exon skipping naïve

Figure 3. Change in mean time from receipt of a prescription to first infusion since the launch of viltolarsen



Compliance

- Patients receiving viltolarsen demonstrated strong compliance by following the prescribed interval and dosing regimen at a rate of 93% from launch to date of analysis

Safety

- The safety profile was similar to the profile observed in clinical trials of viltolarsen

CONCLUSIONS

- This analysis of real-world patient data describes viltolarsen treatment in a broader age range than observed in the clinical trial setting
  - Six percent of patients were younger at viltolarsen initiation than the average age of diagnosis for DMD (4 years old) and younger than participants in all viltolarsen clinical trials[2,3,6,7]
  - The majority of patients (59%) were ≥10 years old at viltolarsen initiation, with the oldest starting at the age of 30, well beyond the age range required for enrollment in viltolarsen clinical trials[2,3]
- The mean age at viltolarsen initiation (12.4 years) was more than 8 years older than the average age of DMD diagnosis[7], which may reflect the various barriers to receiving treatment
- The mean time from receipt of a prescription to the first viltolarsen infusion has substantially decreased since launch and was fastest for young patients and those who switched from a prior exon skipping therapy
- Treatment compliance was strong in patients receiving viltolarsen over the 4 years since launch
- Limitations of this analysis include potential bias introduced from patient-reported information, as well as generalizability to the overall population of patients with DMD who may be eligible for treatment with viltolarsen, as this analysis only included patients who had access to and went on to receive viltolarsen

References

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Disclosures

All authors report employment for NS Pharma, Inc.

Acknowledgments

Medical writing and editorial support were provided by Steven Walker, PhD, of Red Nucleus, and were funded by NS Pharma, Inc.

Presented at the 2025 MDA Annual Meeting; March 16–19; Hilton Anatole, Dallas, TX.