1. Background

- Duchenne muscular dystrophy (DMD) is a severe, late-onset neuromuscular disorder affecting approximately 1 in every 3600–6000 live male births.1–6

- During the 1960s and 1970s, both DMD and muscular dystrophies were treated with corticosteroids, which showed a beneficial effect on muscle function and lung function.7,8

- The ability to walk independently is a sensitive end point to assess DMD disease progression.9

- Most DMD clinical trials have focused on ambulatory patients (defined as patients who can walk unaided for at least 10 m).10

- The long-term effectiveness of ataluren in patients who are no longer ambulatory remains unclear.

- The STRIDE Registry is a matched-cohort study designed to investigate the long-term effectiveness and safety of ataluren in non-ambulatory patients with DMD, to identify any persistent or evolving signs of disease progression.

2. Methods

Study design

- The STRIDE Registry is a post-approval safety study of ataluren use in patients with DMD in routine clinical practice, registered with the European Medicines Agency (EMA).

- The registry can only be implemented in countries where ataluren is commercialized or through access programs.

- It was designed to be implemented in nine countries from 2006 to 2016.9,10

- The registry includes patients with genetically confirmed nmDMD aged 2–28 years who are non-ambulatory at study entry.

3. Results

3.1 Patients

- As of the latest data cut-off date of January 31, 2019, 22 patients were included in the STRIDE Registry, all of whom had received ataluren for ≥ 6 months.

- All patients were non-ambulatory at study entry.

- The majority of patients (86.4%) were in the 10–20 years age range (excluding treatment-naive patients), a years

- Of 210 patients with genetically confirmed nmDMD in the effectiveness population, 18.2% (4/22) had a predicted FVC of < 60%, whereas 54.5% (12/22) had a predicted FVC of < 50%.

- Future comparisons of data from the STRIDE Registry with CINRG DNHS data will provide further real-world insights into the long-term effectiveness of ataluren for the treatment of patients with DMD.

Demographics and characteristics of propensity-score matched non-ambulatory STRIDE Registry and CINRG DNHS patients

- Of the 22 STRIDE Registry patients, 22 were eligible for propensity-score matching and had an available dataset at the age of 18 years (median age of patients in the CINRG DNHS was 19.0 years (range 13.0–28.8 years).

- The study design is shown in Figure 1.

4. CONCLUSIONS

In propensity-score matched analyses of non-ambulatory patients from the STRIDE Registry and CINRG DNHS, STRIDE patients received ataluren for a mean of 302 days before loss of ambulation, which occurred 1.3 years later than CINRG DNHS patients (not yet reported).

The CINRG DNHS and STRIDE registries are ongoing studies that are designed to investigate the long-term effectiveness and safety of ataluren in non-ambulatory patients with DMD. The findings of this analysis are consistent with those observed in previous clinical trials and provide further real-world insights into the long-term effectiveness of ataluren for the treatment of patients with DMD.