AGE AT LOSS OF AMBULATION IN PATIENTS WITH DMD FROM THE STRIDE REGISTRY AND THE CINRG NATURAL HISTORY STUDY: A MATCHED COHORT ANALYSIS

MERCURI, Eugenio[1]; SANTOS, Ana Paula Carneiro[2]; MACHADO, Daiana[2]; FEDERHEN, Andressa[2]; MCDONALD Craig[3].

[1]Department of Pediatric Neurology, Catholic University, Rome, Italy; [2]PTC Farmacêutica do Brasil LTDA, São Paulo, Brazil; [3]University of California Davis School of Medicine, Davis, CA, USA.

Introduction and objective: We examined if nonsense mutation Duchenne muscular dystrophy (nmDMD) patients receiving ataluren plus standard of care (SoC) in the Strategic Targeting of Registries and International Database of Excellence (STRIDE) (NCT02369731) experienced a delay in age at loss of ambulation (LOA) versus DMD patients receiving SoC alone in the Cooperative International Neuromuscular Research Group (CINRG) Duchenne Natural History Study (NCT00468832).
Methods: STRIDE is an ongoing registry providing data on ataluren use in nmDMD patients in routine clinical practice. Data were extracted on January 31, 2022. Propensity score matching identified STRIDE and CINRG patient cohorts (N=260) comparable in predictors of disease progression: age at first symptoms; age at initiation of corticosteroid use; duration of deflazacort use; and duration of other corticosteroid use. Kaplan–Meier analyses were used to estimate age at LOA.
Results: The mean ages at first symptoms in the STRIDE and CINRG were 2.8 years. Most patients (STRIDE vs CINRG) received corticosteroids for ≥12 months (85.0% vs 83.8%), with a similar proportion receiving deflazacort (47.7% vs 44.2%) or other corticosteroids (41.9% vs 43.5%). In the STRIDE cohort, 26.5% (69/260) of patients lost ambulation compared with 54.6% (142/260) of patients in the CINRG cohort. The median ages at LOA (STRIDE vs CINRG) were 17.9 and 12.5 years, respectively. Kaplan–Meier analyses showed that ataluren plus SoC delayed age at LOA compared with SoC alone ($p<0.0001$).
Conclusion: Routine clinical practice ataluren plus SoC delayed age at LOA by 5.4 years compared with SoC alone in patients with nmDMD.

Palavras-chave: Duchenne muscular dystrophy. Nonsense mutation. Ambulation.