PULMONARY FUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY FROM THE STRIDE REGISTRY AND CINRG NATURAL HISTORY STUDY: A MATCHED COHORT ANALYSIS

TULINIUS, Már[1]; SANTOS, Ana Paula Carneiro[2]; MACHADO, Daiana[2]; FEDERHEN, Andressa[2]; MCDONALD, Craig[3]

[1]Department of Pediatrics, Gothenburg University, Queen Silvia Children's Hospital, Gothenburg, Sweden; [2]PTC Farmacêutica do Brasil LTDA, São Paulo, Brazil; [3]University of California Davis School of Medicine, Davis, CA, USA.

Introduction and objective: We investigated 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 lesser decline in pulmonary function versus DMD patients receiving SoC alone in the Cooperative International Neuromuscular Research Group (CINRG) 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 ages at %- predicted forced vital capacity (FVC) <30%.
Results: The mean age at onset of first symptoms in both STRIDE and CINRG were 2.8. 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%). Median (95% confidence interval [CI]) ages at %-predicted FVC <60% (STRIDE vs CINRG) were 17.7 (16.8, not estimable) and 15.3 (14.9, 16.5) years, respectively (p=0.0053). Median (95% CI) ages %- predicted FVC <30% (STRIDE vs CINRG) were not estimable and 22.5 (20.3, 25.4) years, respectively (p=0.0008).
Conclusion: These interim registry data suggest that treatment with ataluren and SoC in routine clinical practice slows disease progression in pulmonary function in nmDMD patients.

Palavras-chave: Duchenne muscular dystrophy. Nonsense mutation. Pulmonary function.