

## One-year shifts in North Star Ambulatory Assessment items in Duchene muscular dystrophy (DMD) patients in the UK NorthStar Network database

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**Background:** The North Star Ambulatory Assessment is a 17 item measure of motor function used in ambulant boys with DMD. Characterizing changes across NSAA items can provide insight into how functional abilities change with age and disease progression, and inform endpoint selection and trial design.

**Objective:** To characterize probability of worsening, improvement or no change in NSAA items among patients stratified by age or functional status.

**Methods:** Patients analyzed were boys with DMD from the UK NorthStar database who had serial NSAA data. All pairs of NSAA assessments spanning up to 16 months were studied, with each pair classified as showing worsening, improvement or no change in item scores. Annualized probabilities of change were estimated using Poisson regression with generalized estimating equations. Analyses were stratified by age and level of function.

**Results:** Data from 357 boys were analyzed. The largest probabilities of improvement were among boys younger than 7 years, for lifting head (25%), hopping (24%), standing on one leg (23-24%) and standing on heels (23%). The largest probabilities of worsening were observed among boys with NSAA <12 for standing on one leg (40-45%), standing (35%), sit to stand (31%) and walk (28%). The probability of shifting up or shifting down were similar for hop in boys under 7yrs, and for run, walk and stand in older boys. Shift up in hop was most probable in high function groups with high function at baseline (10mwr <6s, rise from supine < 4s, NSAA < 20) and NSAA (>20) while probability of shift down was high when rise from supine function was intermediate (4-8s). Shifts down in climb/descend, run and jump were most probable when baseline function was intermediate (10mwr 6-10s; NSAA 12-20) or poor (rise from supine >8s).

**Conclusions:** Stratifying patients by initial function rather than by age is a more suitable approach to identify patients that are likely to have a modifiable decline in NSAA items.

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