Early signs of gross motor delay in very young boys with Duchenne muscular dystrophy
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Objective: This study aims to 1) examine the utility of the Gross Motor portion of the Bayley Scales of Infant and Toddler Development, Third Edition (Bayley-III), North Star Ambulatory Assessment (NSAA), and 100 Meter Timed Test (100m) for use in the very young Duchenne muscular dystrophy (DMD) cohort, 2) confirm differences in motor performance between young boys with DMD and typically developing peers and 3) document natural history and average values in young boys with DMD.

Background: In the era of evolving therapies for the treatment of DMD, outcome measure selection is of utmost importance. Preclinical and early clinical trial data strongly suggest that early treatment will maximize benefits. With the historic average range of diagnosis between the ages of 3-5 years, the differentiation between healthy infants and those with DMD has not been established in infants and young children. Similarly, the magnitude of a change in development different from natural history to document improvements is not as well defined as the older cohorts.

Design/Methods: 114 boys with a confirmed diagnosis of DMD, 0.8–7.9 years (Mean 4.7±1.5), were evaluated using the Bayley-III (N=46, 0.8–5.9 years), NSAA (N=107, 2.5-7.9 years) and 100m (N=74, 3.5–7.9 years) during clinic visits.

Results: Bayley-III scaled scores were lower in boys with DMD compared to published controls (Mean 4.7 ± 1.9; typical peers 10 ± 3). 100m times were significantly lower than age/size matched peers across the age range (Mean 53.4% of predicted). Difficulty with key skills were indicative of early gross motor involvement. The delay in gross motor skills remained present over time.

Conclusions: Gross motor delay can be measured in infants and young boys with DMD using the Bayley-III, NSAA, and 100m. Reference values by age and steroid regimen will be presented to document the natural history.