Longitudinal Changes in the North Star Ambulatory Assessment and Leg Muscle Fat Fraction in DMD

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INTRODUCTION

- North Star Ambulatory Assessment (NSAA) is a functional assessment scale validated for ambulatory individuals with Duchenne muscular dystrophy (DMD).[1,2]
- NSAA has been utilized to assess disease progression and efficacy of interventions in DMD.[3,4]
- Furthermore, proton magnetic resonance spectroscopy (^{1}H-MRS) measures of fat fraction are reproducible, correlate with clinical functional tests, and are sensitive to disease progression and treatments in DMD.[5-9]
- However, the relationship between NSAA and MR measures of fat fraction is less established.

AIM

- To evaluate the relationship of NSAA and fat fraction measures of lower extremity muscles in a longitudinal study of DMD.

METHODS

Subjects: A total of 90 ambulatory subjects with DMD (age 4-18 years at baseline) from the ImagingNMD project were included in this study, with time points acquired yearly for up to 3 years.

MR data:

- Single voxel ^{1}H-MRS data were acquired at 3T using stimulated echo acquisition mode (STEAM) with relaxation corrections to measure fat fraction from the soleus (Sol) and vastus lateralis (VL; Figure 1).[5-9]

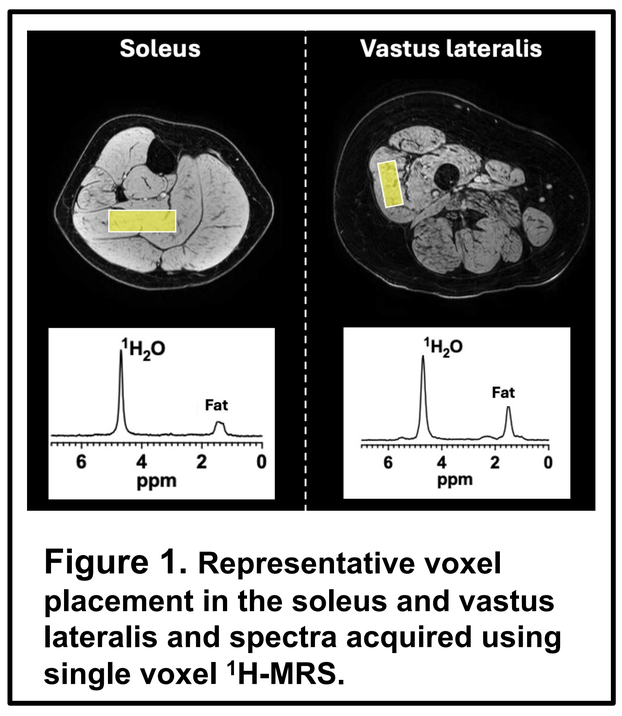

Figure 1. Representative voxel placement in the soleus and vastus lateralis and spectra acquired using single voxel ^{1}H-MRS.

Functional data:

- NSAA total score was calculated as the sum of scores on 17 ordinal tests, scored as 0, 1, or 2, with a 2 indicating normal activity.[1]
- Relationships among measures were evaluated using non-parametric correlation and probability analyses.

RESULTS

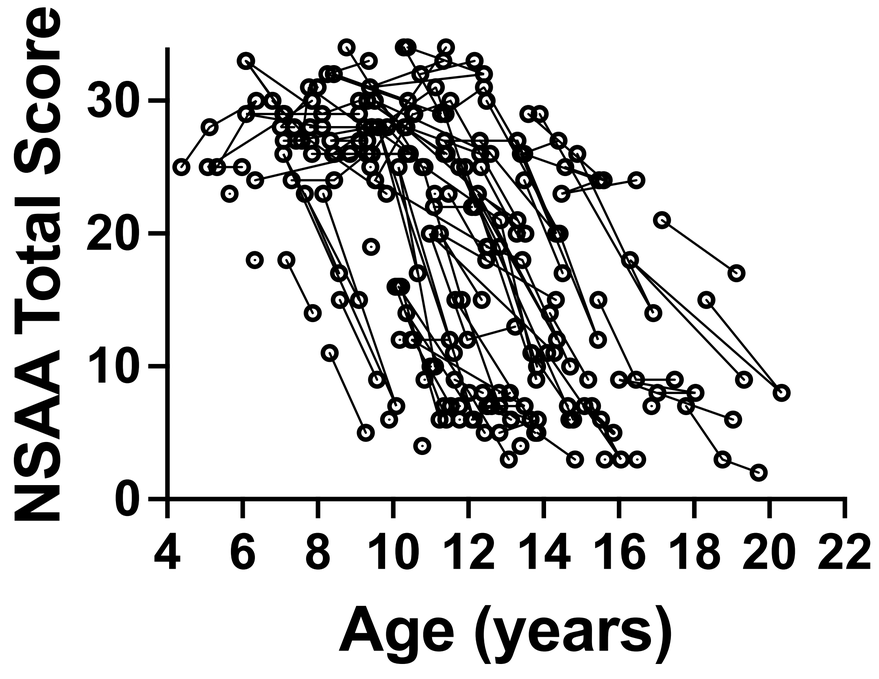

Figure 2. Longitudinal North Star Ambulatory Assessment (NSAA) total scores in individuals with Duchenne muscular dystrophy (DMD) plotted relative to age.

RESULTS (Continued)

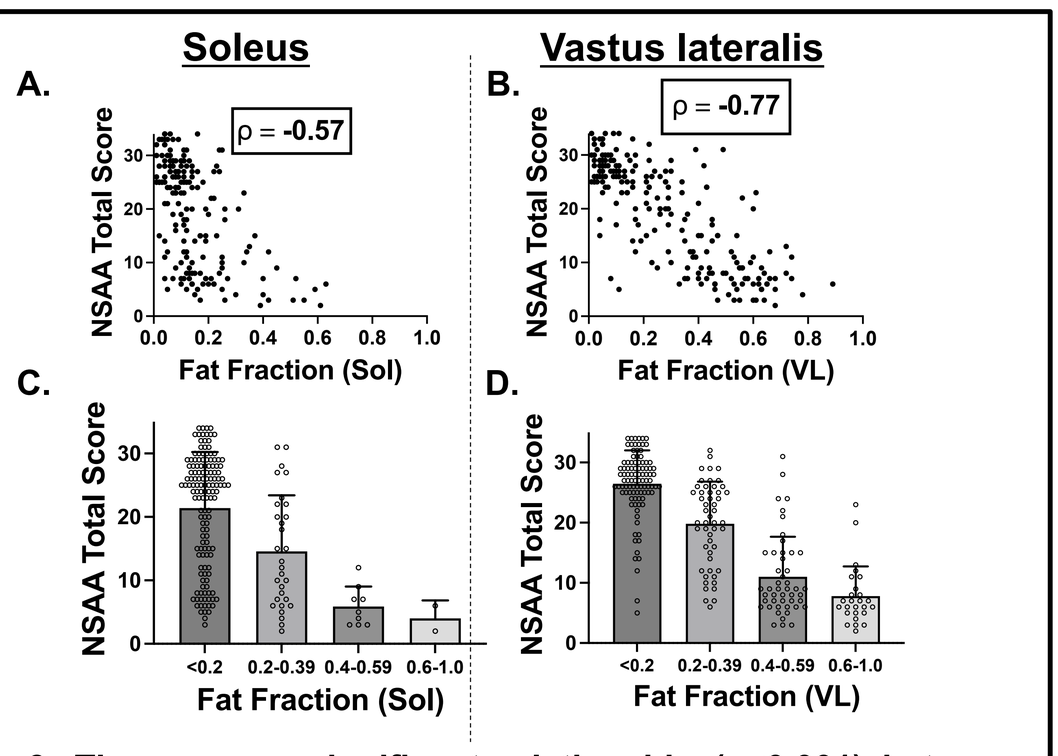

Figure 3. There was a significant relationship (p<0.001) between NSAA total score and fat fraction of the soleus (A) and vastus lateralis (B). Furthermore, with increasing fat fraction categories, there was, on average, a drop in NSAA total score (C,D).

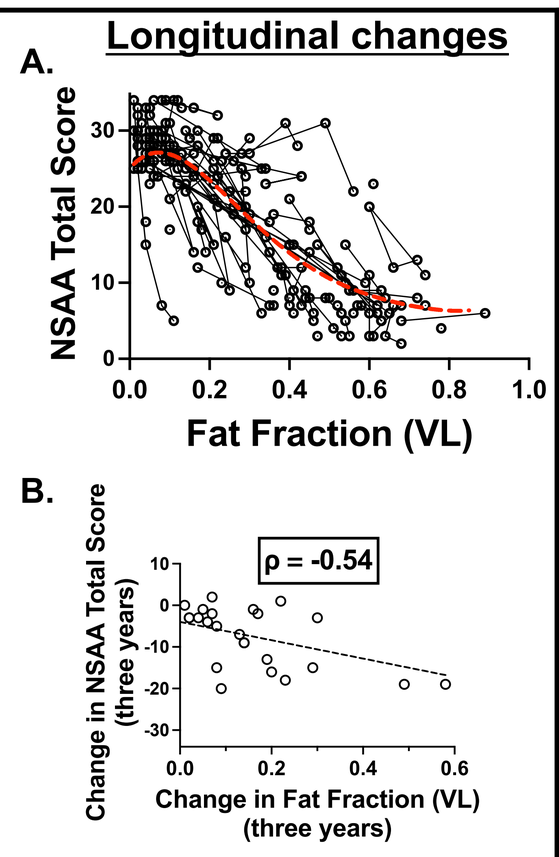

Figure 4. NSAA total score tended to increase at low fat fraction (less than ~0.1), then decline with increasing fat fraction (A). Three-year longitudinal changes in fat fraction correlated with NSAA total score changes (p<0.01; B).

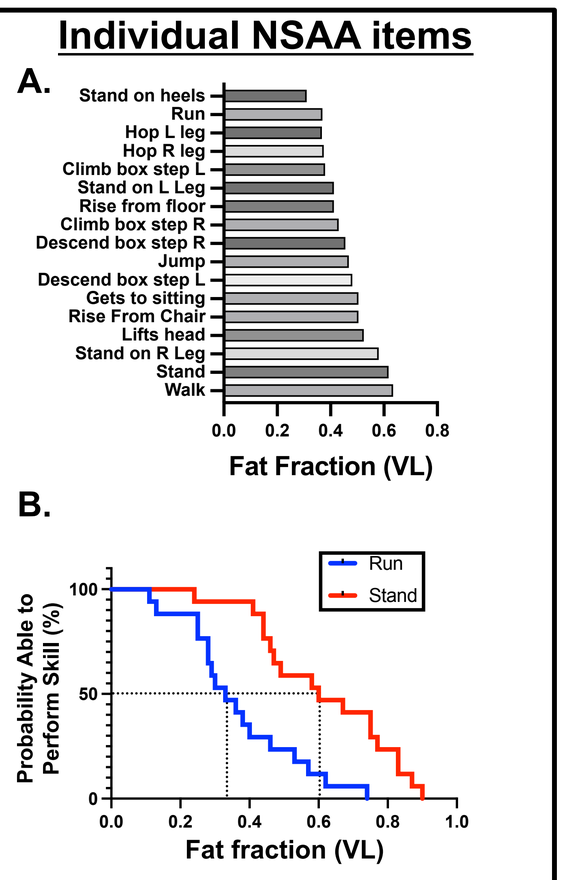

Figure 5. The loss of ability to perform the more difficult tasks in the NSAA occurred at a lower fat fraction (A). Kaplan-Meier plot for loss of ability to stand and run in relation to VL fat fraction in DMD (B).

CONCLUSIONS

- Overall, NSAA was strongly correlated with muscle fat fraction determined by ^{1}H-MRS, and our findings are consistent with the notion that muscle fat fraction can be used to predict functional abilities included in NSAA.

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