

## **REAL-WORLD ASSOCIATIONS BETWEEN MOTOR FUNCTION AND LEAN BODY MASS IN THE ARMS AND LEGS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**

Jessica Marden<sup>1</sup>, Jon Freimark<sup>1</sup>, Zhiwen Yao<sup>1</sup>, James Signorovitch<sup>1</sup>, Cuixia Tian<sup>2</sup>, Brenda Wong<sup>3</sup>, Ward SJ<sup>4</sup>, for the Collaborative Trajectory Analysis Project (cTAP)

<sup>1</sup>Analysis Group, Inc., Boston, Massachusetts

<sup>2</sup>Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio

<sup>3</sup>University of Massachusetts Medical School, Worcester, Massachusetts

<sup>4</sup>Collaborative Trajectory Analysis Project, Cambridge, MA, USA

*Email address of presenting author: Brenda.Wong@umassmemorial.org*

Patients with Duchenne Muscular Dystrophy (DMD) have progressive loss of motor function and skeletal muscle mass. This study assessed real-world associations between motor function and % lean body mass in the arms and legs in boys with DMD. Using data from Cincinnati Children's Hospital and Medical Center (CCHMC), 2,514 assessments with data on both the functional motor scale (FMS) and dual energy X-ray absorptiometry (DEXA) lean body mass were available for n=576 patients (ages 2.8-32.5 years). The FMS ranges from 1 to 8, with 8 representing the worst motor function and FMS>4 corresponding to use of a wheelchair as the primary means of mobility. Across all assessments, the mean (standard deviation) for % lean mass was 57.4% (13.8%) in the arms and 55.7% (12.7%) in the legs; correlation between arms and legs was 0.94 (P<0.01). On average, worse motor function was associated with lower % lean mass in arms and legs. E.g., at FMS=1 (i.e., mild abnormalities in gait), the average % lean mass in arms was 68.1% (9.3%) and the average in legs was 66.9% (9.1%); at FMS=4 (i.e., independent walking with a needed means of support), average % lean mass was 50.2% (8.8%) in arms and 46.4% (7.2%) in legs; at FMS=5 (i.e., use of a wheelchair as the primary means of mobility) the average % lean mass was 44.7% (7.9%) in arms and 42.7% (4.9%) in legs; and at FMS=7 (i.e., unable to propel a manual wheelchair) the average % lean mass was 36.9% (7.0%) in arms and 40.7% (5.9%) in legs. Averages for % lean mass in arms and legs tended to be within  $\pm 5\%$  of each other at each level of FMS. Numerically, the average % lean mass in arms was higher than in legs among patients with FMS < 6, but average % lean mass was numerically higher in legs among patients with FMS  $\geq 6$ . Among boys with DMD, percent lean body mass of arms and legs measured using DEXA is correlated with functional decline; DEXA offers a good assay for the study of lean mass changes in patients with DMD.

Word count: **1,974** (WMS max is 2,000 characters including spaces)