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Responsiveness and Minimal Clinically Important Difference of the Motor Function Measure in collagen VI-related dystrophies (COL6-RD) and laminin alpha2-related muscular dystrophy (LAMA2-RD)

Laure Le Goff ¹, Katherine G Meilleur ², Gina Norato ³, Pascal Rippert ⁴, Minal Jain ⁵, Margaret Fink ⁶, A Reghan Foley ⁶, Melissa Waite ⁵, Sandra Donkervoort ⁶, Carsten G Bönnemann ⁶, Carole Vuillerot ⁷

Affiliations

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Abstract

Objective: To investigate the responsiveness of the Motor Function Measure (MFM) and determine the Minimal Clinically Important Difference (MCID) in individuals with two common types of congenital muscular dystrophy (CMD).

Design: Observational, prospective, single center, cohort study.

Setting: National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (NIH).

Participants: 23 individuals with COL6-RD and 21 individuals with LAMA2-RD enrolled in a 4-year longitudinal natural history study.

Interventions: Not applicable.

Main outcome measure: Responsiveness of the MFM-32 and the Rasch-scaled MFM-25 and the MCID of the MFM-32 determined from a patient-reported anchor with 2 different methods: within-patient and between-patient.

Results: Original MFM-32 and Rasch-scaled MFM-25 performed similarly overall in both the COL6-RD and LAMA2-RD populations, with all subscores (D1 standing and transfers, D2 axial and proximal, D3 distal) showing a significant decrease over time, except MFM D3 for LAMA2-RD. MFM D1 subscore was the most sensitive to change for ambulant individuals, whereas MFM D2 subscore was the most sensitive to change for non-ambulant individuals. The MCID for MFM-32 total score was calculated to be 2.5 and 3.9 percentage points according to 2 different methods.

Conclusion: The MFM showed strong responsiveness in individuals with LAMA2-RD and COL6-RD. Because a floor effect was identified more prominently with the Rasch-Scaled MFM-25, the use of the original MFM-32 as a quantitative variable with the assumption of scale linearity appears to be a

good compromise. When designing clinical trials in congenital muscular dystrophies, the use of MCID for MFM should be considered to determine if a given intervention effects show not only a statistically significant change but also a clinically meaningful change.

Keywords: Disability Evaluation; Muscular Dystrophies; Outcome assessment; Responsiveness.

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