

Reliability of the Performance of Upper Limb assessment in Duchenne muscular dystrophy [☆]

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Abstract

The Performance of Upper Limb was specifically designed to assess upper limb function in Duchenne muscular dystrophy. The aim of this study was to assess (1) a cohort of typically developing children from the age of 3 years onwards in order to identify the age when the activities assessed in the individual items are consistently achieved, and (2) a cohort of 322 Duchenne children and young adults to establish the range of findings at different ages. We collected normative data for the scale validation on 277 typically developing subjects from 3 to 25 years old. A full score was consistently achieved by the age of 5 years. In the Duchenne cohort there was early involvement of the proximal muscles and a proximal to distal progressive involvement. The scale was capable of measuring small distal movements, related to activities of daily living, even in the oldest and weakest patients. Our data suggest that the assessment can be reliably used in both ambulant and non ambulant Duchenne patients in a multicentric setting and could therefore be considered as an outcome measure for future trials.

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Keywords: Upper limb; Duchenne muscular dystrophy

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1. Introduction

In the last few years a number of therapeutical approaches have become available for Duchenne muscular dystrophy (DMD). The rapidly increasing number of ongoing or planned clinical trials has highlighted the need to identify reliable outcome measures [1,2]. So far the trials have mainly targeted young ambulant DMD boys [3,4], using the 6 min walk test (6MWT) [5,6] or other functional measures such as timed tests or the North Star Ambulatory Assessment (NSAA). In order to have the possibility to also include non ambulant DMD boys and adults in the forthcoming trials, and, more generally, to have measures that could be used across spectrum of abilities, allowing to follow the boys who may lose ambulation during a trial, there has been increasing attention on assessments of upper limb function [7]. An international Clinical Outcomes Group consisting of clinicians, scientists, patient advocacy groups and industries worked together to develop the Performance of the Upper Limb (PUL), a tool specifically designed for assessing upper limb function in ambulant and non-ambulant DMD patients [8–10]. The development of the PUL was based on a conceptual framework reflecting the progression of weakness and natural history of functional decline in DMD. The DMD boys and their families were involved iteratively throughout the process, providing comments on the relevance of individual PUL items to abilities of daily living and on their clinical meaningfulness. Modern psychometric methods (Rasch analysis) were used to improve robust internal reliability, validity, and hierarchical scalability [10].

All the tasks included in the PUL were selected including activities of daily living that should be performed even by preschool children. We were postulating that typically developing children of 3–4 years of age would pass all the items and complete the scale with a plateau of scores in older children. In order to avoid ceiling effect we also included timed items evaluating the speed to perform some tasks such as stacking cans.

The aim of this cross sectional study was to perform PUL in DMD boys and adults and in typically developing boys and male adults in order to observe the distribution of scores and the suitability of the scale at different ages. More specifically we wished to establish (a) the age when typically developing children are able to complete the assessment (b) the profile of scores in DMD boys and adults at different ages and their relationship with the scores found in typically developing subjects. We also wished to investigate whether the timed items could provide additional value to the scale avoiding possible ceiling effect.

2. Subjects and methods

The study is part of a longitudinal multicentric study aimed at establishing changes in upper limb function in DMD. The study was approved by the Ethics committee

in each centre. As part of this study all the Italian tertiary care centers for neuromuscular disorders consecutively enrolled 322 DMD patients (mean age 12.7; range 4.1–35.1) attending their routine follow up clinics between September 2012 and April 2013.

In order to establish the earliest age when the PUL can be completed, 277 typically developing boys and young adults were also examined by six examiners from 3 centers (Rome, Milan, Pisa) involved in the DMD study. In the first phase we systematically assessed children up to the age of 5, assessing 47 children between 3 and 5 years. Following the observation that there was already a plateau of full scores by 5 years, we also performed the PUL in 230 boys and young adults between the age of 5 and 25 in order to confirm the plateau in subjects older than 5 years and observe possible variations of the timed items with age.

2.1. PUL

The PUL includes 22 items (online appendix) with an entry item to define the starting functional level, and 21 items subdivided into shoulder level (4 items), middle level (9 items) and distal level (8 items) dimension [10]. For weaker patients a low score on the entry item means high level items do not need to be performed. Scoring options vary across the scale between 0–1 and 0–6 according to performance. Each dimension can be scored separately with a maximum score of 16 for the shoulder level, 34 for the middle level, and 24 for the distal level. A total score can be achieved by adding the three level scores (max global score 74).

2.2. Training sessions

At least one therapist from each group attended a first training session where a senior physiotherapist (ESM) presented the PUL with item description, scoring system and demonstrated scale administration through patient videos to outline any scoring shortcomings or issues. All participants were subsequently asked to video a patient assessment. After a review with the senior physiotherapist and the resolution of possible scale administration issues, the 14 clinical evaluators were then asked to score three more videos to outline any difficulties and possible lack of agreement in scores and to determine inter-rater reliability. The results showed an ICC of 0.96.

Three examiners (ESM, LF, RDS) also assessed six children twice with intervals ranging from 1 h to 1 week with identical results between the first and the second assessment in all six.

2.3. Statistical analysis

Non-linear relationships with age were preliminarily assessed by a visual inspection of the plots and then analyzed using a piecewise linear regression. A piecewise

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