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Is one trial enough for repeated testing? Same-day assessments of walking, mobility and fine hand use in people with myotonic dystrophy type 1

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Abstract

Performance-based assessments of physical function are essential in people with myotonic dystrophy type 1 (DM1) to monitor disease progression and evaluate interventions. Commonly used are the six-minute walk test, the 10 m-walk test, the timed up-and-go test, the timed-stands test, grip strength tests and the nine-hole peg test. The number of trials needed on a same-day test occasion and whether the first, best or average of trials should be reported as result is unknown. Thus, the aim was to describe and explore differences between trials in these measures of walking, mobility and fine hand use in 70 adults with DM1. Three trials were performed for each test except for the six-minute walk test where two trials were allowed. There were statistical significant differences over trials in all tests except for the 10 m-walk test and grip strength tests. Pair-wise comparisons showed that the second and third trials were in general better than the first, although effect sizes were small. At which trial the individuals performed their best differed between individuals and tests. People with severe muscular impairment had difficulties to perform repeated trials. Intraclass correlation coefficients were all high in analyses exploring how to report results. The conclusion and clinical implication is that, for a same-day test occasion, one trial is sufficient for the 10 m-walk test and grip strength tests, and that repeated trials should be allowed in the timed up-and-go test, timed-stands test and nine-hole peg tests. We recommend that two trials are performed for these latter tests as such a protocol could accommodate people with various levels of impairments and physical limitations.

Keywords: Myotonic dystrophy; Outcome measures; Reliability; Number of trials; Repeated measures; Rehabilitation

1. Introduction

Myotonic dystrophy type 1 (DM1), with an estimated worldwide prevalence of 5–20 per 100,000 [1], is one of the most common neuromuscular diseases. It is inherited in a dominant pattern, and caused by an unstable CTG repeat expansion on chromosome 19 [2] and with accumulating evidence for being RNA-mediated [3]. Characteristic symptoms are myotonia and muscle weakness with a distal-to-proximal progression order in limb muscles [4]. The disease is multi-systemic affecting ocular, cardiovascular, respiratory, digestive, metabolic, endocrine, and central nervous system functions [1]. There is a wide inter-individual variety both in muscle and in other system involvement, and in progression rate.

Top-priorities for patients with neuromuscular diseases are research on finding a cure, slowing down progression and prevention of occurrence of symptoms [5]. There is no cure available for DM1, although there is reason to believe that gene therapy will be a possibility in the relatively near future [6,7]. A main target will be to improve muscle function in order to improve performance of activities in daily living, social participation and quality of life [8]. However, concerns about

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clinical endpoints and metrological sound outcome measures have been raised from clinical, scientific and patient perspectives [9–11]. There is a need to provide the DM1 community with relevant outcome measures and to achieve international consensus on which ones to use. To answer some of these issues, an international collaboration has been ongoing since 2011 called "Outcome Measures in MYotonic Dystrophy" (OMMYD) [12,13]. A variety of performance-based measures of physical function have been discussed and proposed at these workshops. However, there has been a debate on the number of trials that need to be performed on each test occasion and, if more than one trial is needed, on how to report the result (first, best or the average of trials) with regard to reliability of the outcome measures. That is, if a patient's and/or rater's behaviour is consistent under given conditions, then measurements should be reproducible, i.e. reliable and free from errors. However, in reality, there are always some errors attributed to measurements which affect a patient's score from trial to trial. This is especially true in DM1 where errors might occur from factors such as cognitive impairments, fatigue [14], apathy behaviour and lack of motivation [15], but also due to potential learning effects. Thus, the aim of the present study was to describe and explore differences between repeated trials from the same-day test occasion in performance-based measures of walking, mobility and fine hand use in people with DM1.

2. Materials and methods

2.1. Participants

Participants were mainly recruited from the Department of Neurology at the Karolinska University Hospital, Stockholm, Sweden, as previously described [16,17]. Inclusion criteria were adults, i.e. ≥18 years old, with a genetically confirmed diagnosis of DM1 who were living in the Stockholm County Council. Thirty-three of 107 identified eligible people declined participation and four did not reply, thus, 70 people with DM1 were included in the study. All participants gave their signed informed consent before enrolment. The study was approved by the Regional Ethical Review Board in Stockholm and procedures were conducted in accordance with the Helsinki Declaration.

2.2. Measures and procedures

Data on gender, age, hand dominance, weight and height were collected for descriptive purposes. The DM1-specific muscular impairment rating scale (MIRS) [18,19] was used to describe major stages of disease severity with regard to muscular impairments. The scale is based on manual muscle testing of the neck flexors and six proximal and four distal muscle groups bilaterally. The five-point scale ranges from no muscular impairment to severe proximal weakness. Mild muscular impairment was defined as MIRS grades 1–3 and severe as MIRS grade 4–5.

The 10 m-walk test (10mWT) [20] and the six-minute walk test (6MWT) [21] were used for assessment of walking capacity. For the 10mWT, persons were instructed to walk 20 m, on a flat floor in a corridor, as fast as possible. Timing with a handheld stopwatch was done over the middle 10 m of the 20 m walkway to avoid measuring acceleration and deceleration. Gait speed was calculated for each of three possible trials. The 6MWT was administered according to the American Thoracic Society (ATS) guidelines [22]. A 30-m track on a flat floor in a corridor was used and the turnaround points at the beginning and end of the track were marked by orange plastic cones. The instruction was to walk as far as possible during the test. In accordance with the ATS guidelines, standardized encouragement and information about the time left was provided regularly during the test. After six minutes, the person was told to stop, and the distance walked was measured. For the 6MWTs, considering time length, participants performed only two trials on the same day approximately one hour apart. Use of walking aids and/or orthosis during walking tests was documented.

The timed-stands test (TST) [23] and the timed up-and-go test (TUG) [24] were used for assessment of mobility. The TST records the time it takes for a person to rise and sit down 10 times as quickly as possible, without using the arms, from a chair without armrest (45 cm seat height). Participants kept their arms alongside the body and a full stand was practised before the first trial. The TUG records the time taken to stand up from a chair with armrest (45 cm seat height), walk three metres, turn, walk back and then sit down. In TUG, participants were instructed to rise on the word "go" and to walk at their preferred self-selected speed. Timing was begun when their back left the back of the chair and stopped when the buttocks touched the chair seat again. Three trials were performed for each of the mobility tests.

Grip strength tests and the nine-hole peg test (NHPT) were used for assessment of fine hand use. Following the standardized instructions by Mathiowetz et al. [25,26], grip strength was assessed by use of a standard hydraulic hand dynamometer (ref 43050, Chattanooga Group USA). Participants were tested in a seated position with the elbow in 90 degrees flexion. Verbal encouragement was given during the task. Three trials were performed for each hand, with a 10–15 s rest between trials. The NHPT records the time taken to place nine pegs in a board and then remove them again as quickly as possible. The test equipment and the standardized procedures as described by Mathiowetz et al. [27] were used. Three trials were performed for each hand with at least 10–15 second rest between trials.

All tests were administered by the same physiotherapist (MK). The order and instructions were standardized according to a test protocol.

2.3. Statistical methods

Fisher's exact test and independent t-tests were used to analyse differences in sex and age, respectively, between those included and those not participating in the study. Descriptive statistics were used to present data, i.e. frequency and percentage, median and interquartile range (IQR), and mean and standard deviation (SD). Normal distribution was evaluated by descriptive statistics (histograms, normality plots), Kolmogorov–Smirnov and Shapiro–Wilk tests. Generalized Estimating Equations (GEE) with an unstructured correlation matrix were used to explore differences between trials. Analyses were run using both linear and gamma distribution and the model with the lowest QICC