S.P.2
Disease progression as measured by the 6MWD in Duchenne Muscular Dystrophy; a single centre experience of 62 boys treated with daily corticosteroids
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The 6-min walk test (6MWD) is an accepted method to assess ambulatory capacity in Duchenne Muscular Dystrophy (DMD) patients and used as a primary endpoint in therapeutic trials. Objectives: To describe the 6MWD by age and its change over time in a homogenous population of corticosteroid treated DMD boys receiving a similar standard of care. This assessment reports functional test data, collected as part of the clinical management at a single centre, excluding subjects involved in investigational trials, non-steroid treated DMD and Becker patients. Sixty-two boys were identified, mean 9.8 years (range 5.1 and 17.8 at first observation). 6MWD was described for 1 year age groupings, an average 6MWD was calculated for any boy with multiple measurements within age groups. In addition changes in 6MWD at approximately 1, 1.5 and 2 years (±10 weeks) of follow up were evaluated. 6MWD showed improvement from age group 4.5–5.5 years (Mean 357 m, SD 30, Median 369) to age group 6.5–7.5 years (450 m, 86, 427) followed by a decline, which became precipitous from 12.5 years onwards. From 15.5 years all boys were unable to perform the 6MWD. Changes in 6MWD demonstrated a median (mean, SD) decline of −10 m (−38, 96) at 1 year (N = 19, mean baseline age 9.7 years), −27 m (−38, 58) at 1.5 years (N = 8, mean baseline age 10.2 years) −125 m (−129, 129) at 2 years (N = 13, mean baseline age 10.1 years). This study provides substantial homogenous data in 62 DMD boys treated with daily corticosteroids on the ambulatory capacity of DMD and its changes over time. The age-related aspects and the slope of decline of the 6MWD should be considered in the design and interpretation of therapeutic trials in ambulant DMD patients.

http://dx.doi:10.1016/j.nmd.2012.06.241

S.P.3
Diverse walking distances predict functional outcomes in Duchenne Muscular Dystrophy
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Evaluate the utility of timed walking tests in DMD. Many different timed walking tests have been shown to be accurate, reproducible, and simple to administer (10 m walk (10 mw), 2 min (2MWT), and 6 min (6MWT)). Recently the 6MWT has emerged as the gold standard for use in clinical trials. Thirty five subjects with DMD were evaluated 95 times. Tests include walking distance; isometric quadriceps strength testing and the North Star Ambulatory Assessment (NSAA). Mixed model analysis revealed that both the 2MWT and 6MWT are highly significant correlations to the NSAA (p = 0.0001) and quadriceps strength (p = 0.05). Boys walked a consistent speed throughout the entire test. Velocity during each minute of a single session ranged from 0.97 to 1.06 m/s, showing a high degree of correlation at each timed interval (r = 0.725 – 0.921; p < 0.001). Wilcoxon signed rank tests on walking test group data revealed no significant difference between distances on day 1 and day 2 for either the 2MWT (p = 0.8) or the 6MWT (p = 0.5). Individual variability between days, however, was quite large. For 6MWT distance walked between subsequent days ranged from 0–44 m or 0–59% (mean = 6.4% = /− 10.26) of the total distance. Variability for the 2MWT was similar, ranging from 0–33 m or 0–56% (mean = 7.9% = /−10.05). In DMD the 2MWT is comparable to the 6MWT. Both correlate with quadriceps strength and function. The caveat for the data set analyzed here is that each minute was part of a 6MWT. Future studies will need to test walking velocity in separate designated 6 and 2 min tests.

http://dx.doi:10.1016/j.nmd.2012.06.242

S.P.4
Functional changes in Duchenne muscular dystrophy: A 24 month longitudinal cohort study
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Six minute walk test (6MWT), timed items and North Star Ambulatory Assessment (NSAA) are increasingly used as possible outcome measures in clinical trials in Duchenne Muscular Dystrophy (DMD). Longitudinal data have previously been reported following changes in their scores over a 12 month period. The aim of the study was to assess 6MWT and NSAA in a cohort of 119 ambulant DMD boys over 24 months in order to establish the spectrum of possible changes over a longer period of time. The study is a longitudinal multicentric cohort study. 119 ambulant DMD patients were assessed using 6MWT, NSAA at baseline 12 and 24 months. Clinical data including age and steroid treatment were collected. During the 24 months of the study, we observed a progressive decline in both measures that was more obvious in the second year. Not all the DMD boys in our cohort showed a decline as young boys showed some improvement in their 6MWT and NSAA scores up to the age of 7. Fifteen patients (12.6%) lost the ability to walk independently: 2/15 by the end of the first year and the other 13 in the second year. Another 22 patients (21.1%) were still able to walk independently but were unable to get up from supine (8/22 at baseline, 4 at 12 months, 10 at 24 months). Four children also lost the ability to perform the 6MWT (2 at 12 months and the other 2 at 24 months). This study provides longitudinal data of NSAA and 6MWT over a 24 month period. These data can be useful when designing a clinical trial.

http://dx.doi:10.1016/j.nmd.2012.06.243

S.P.5
Trial readiness: Clinical interpretability of change scores of the North Star Ambulatory Assessment in Duchenne muscular dystrophy
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Evaluate the utility of the North Star Ambulatory Assessment (NSAA) as a tool to measure disease progression in Duchenne muscular dystrophy (DMD). The NSAA is a standardised, easy to administer, test of ambulatory function that has been in use in clinical trials for 15 years. The purpose of this analysis is to determine its interpretability as a clinical tool in order to validate its use as a clinical tool in order to validate its use in clinical trials. The study is a longitudinal multicentric cohort study. 119 ambulant DMD patients were assessed using 6MWT, NSAA at baseline 12 and 24 months. Clinical data including age and steroid treatment were collected. During the 24 months of the study, we observed a progressive decline in both measures that was more obvious in the second year. Not all the DMD boys in our cohort showed a decline as young boys showed some improvement in their 6MWT and NSAA scores up to the age of 7. Fifteen patients (12.6%) lost the ability to walk independently: 2/15 by the end of the first year and the other 13 in the second year. Another 22 patients (21.1%) were still able to walk independently but were unable to get up from supine (8/22 at baseline, 4 at 12 months, 10 at 24 months). Four children also lost the ability to perform the 6MWT (2 at 12 months and the other 2 at 24 months). This study provides longitudinal data of NSAA and 6MWT over a 24 month period. These data can be useful when designing a clinical trial.

http://dx.doi:10.1016/j.nmd.2012.06.243
The North Star Ambulatory Assessment (NSAA) is a clinician-rated measure of ambulation in Duchenne muscular dystrophy (DMD). A key role of the NSAA is to monitor disease progression and treatment impact (e.g. steroids). However, to date, the responsiveness (i.e., ability to detect change) of the NSAA has not been determined. The aim of this study was to address this issue. Data was collected on boys with DMD from the UK North Star clinical database (n = 804 measurements). Two subgroups of patients were defined: daily versus intermittent prednisolone steroid regimes. The NSAA was transformed to 0–100 range based on Rasch measurement methods. The responsiveness of the NSAA was examined through mean change scores over time, pair-wise squared t-values from paired samples t-tests, and effect size (ES) calculations. Minimal Important Difference (MID) statistics were derived based on distribution-based indicators. Data were analysed using RUMM2030 and SPSS-19. Generally, mean NSAA scores were higher in the daily group (mean change (SD) range: 52.9 (24.4) to 73.6 (13.6)) compared to the intermittent group (mean change (SD) range: 46.2 (14.5) to 68.9 (12.9)). The associated responsiveness statistics for the daily group suggested consistent low/moderate changes (mean change (SD) range: −7.5 (12.2) to 13.8 (17.6); ES range: 0.15 to 0.67) over time. For the intermittent group this fluctuated between low, moderate and large changes (mean (SD): −7.2 (8.5) to 13.8 (11.9), ES: 0.02 to 1.01). The mean MID was 8.8 and 6.9, for PF Regime_1 and PF Regime_2, respectively. In this first study to explore the responsiveness of the NSAA in DMD, the findings support the ability of the NSAA to detect important change over time. In addition our initial proposed MIDs provide a start point to provide clinical meaning of changes scores over time. This study also further demonstrates the importance of using Rasch measurement methods to better understand the development and use of rating scales.

http://dx.doi:10.1016/j.nmd.2012.06.244

S.P.6 Exploratory Rasch analysis of the EK2 scale used in a population of Duchenne muscular dystrophy (DMD)
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The Egen Klassifikation scale was designed to measure functional ability in non-ambulant DMD. It examined activities and abilities such as transfers, trunk mobility, wheelchair use, bed mobility, cough, well-being, and the original scale of 1–10 items showed good validity and reliability using traditional methods of psychometric analysis. The scale was extended in 2008 by adding seven items to capture functional ability specifically related to feeding, bulbar issues and distal hand function. At the same time items 1–10 were revisited to update the scale in relation to advances in equipment and respiratory management. The aim of this study was to re-analyse the revised and extended scale (1–17 items) by traditional methods in this larger cohort of patients, and then to apply more rigorous psychometric analysis to examine uni-dimensionality, item fit, reliability and dependency. Over a period of 4 years, data were collected during regular clinic visits by trained clinical evaluators (two from Denmark and four from Newcastle). A cross-sectional Danish cohort of non-ambulant men with DMD, n = 83, age range 18–46 and a younger population of non-ambulant boys and men from Newcastle UK, n = 70, age range 11–37 (total 153, age range 11–46), were examined for psychometric properties including clinical meaning, targeting, response categories, fit, reliability, dependency, stability and raw to interval level measurement. All analyses were performed in RUMM2030. Psychometric analysis showed strengths and weaknesses within the scale. The EK scale is clinically relevant enabling clinicians to focus on practical issues and highlighting areas of concern. However it is uncertain whether the scale has sufficient sensitivity for application in clinical trials or whether the additional items are beneficial. This current collaborative work will help us to clarify these issues.

http://dx.doi:10.1016/j.nmd.2012.06.245

S.P.7 Validation trial of a movement Holter monitor based on accelerometer for the non-ambulatory neuromuscular patients
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One of the most important and challenging issues regarding the clinical trials in non-ambulatory neuromuscular patients is the lack of a reliable and reproducible evaluation method, sensitive enough to quantitatively assess the function of the upper limbs of disabled patients. A movement Holter monitor composed of tri-axial accelerometer, gyroscope and magnetometer is being developed in France at the Institute of Myology in collaboration with SYSNAV Company. The measuring principle is based on inertial sensors. The device is sufficiently light and non-intrusive to be worn as a watch and has 18 h of autonomy. A validation trial of a wireless version of the system is ongoing in 10 non-ambulant neuromuscular patients. The upper limb function of the patients wearing the device is evaluated during a series of different filmed tasks. The evaluations take place at the Institute of Myology at the day of the inclusion and are repeated 14 days after. The system contains a second set of inertial sensors placed on the wheelchair in order to detect its movement. Between the two visits, the patients are continuously recorded in their own environment during daytime. The measurements were feasible even in a very weak 29 year-old Duchenne patient. The data permit to explore new variables representing the upper limb activity level and quantify specific movements. In a preliminary phase, the orientation of the wrist and periods of activity could be identified during a given period of time. Subsequent analyses will assess the identification, characterization and quantification of movements of the wrist taking into account the wheelchair movement. The novelty of this project resides in the possibility of a quantitative evaluation of non-ambulatory neuromuscular patients with limited abilities both in a standardized setting and in their natural environment, thanks to the high-autonomy of this wireless device and its high accuracy.

http://dx.doi:10.1016/j.nmd.2012.06.246

S.P.8 Validation of linear accelerations and angular velocities to estimate the efficacy of a subject when performing a quantified task in a controlled environment
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The need for measures specifically designed to assess the muscle strength and activity of upper limbs of non-ambulatory patients with neuromuscular...