Neuro-muscular diseases

Oral communications

CO019
The motor function measure (MFM) in the myotonic dystrophy type 1 population: Description and responsiveness
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Objective To assess the applicability and the responsiveness of the motor function measure 1 (MFM) in the myotonic dystrophy type 1 (DM1) population.

Materials/patients and methods We conducted an observational, retrospective, multicenter cohort study using data from the MFM database (http://www.motor-function-measure.org/data-bank.aspx). Only DM1 patients with at least one MFM-32 score were included. The distributions of the MFM scores (total score and 3 subscores) were analyzed by age. MFM responsiveness was estimated in patients with at least two MFMs (at least six months between the two evaluations). Hypothetical sample sizes for specific effect sizes in clinical trial scenarios are given.

Results The descriptive study includes 618 patients from 29 physical medicine and rehabilitation or neurology department aged 6.2–80.4 years. 1038 MFM-32 for DM1 patients are registered in the MFM database and 228 patients have at least two evaluations. 930 MFM were realized in adults’ patients (> 18 years old). Mean age at the MFM execution was:

- MFM D1 subscore (standing and transfers) is the more sensitive score to show deterioration (~2.32 points/year).
- MFM D2 (proximal and axial motricity) and D3 (distal motricity) subscores showed less changes over time (~1.43 + –4.25 points/year for D2, ~0.53 + –4.18 points/year for D3). Significant responsiveness was obtained with the D1 subscore (standardized response mean [SRM] = .550).

Discussion/Conclusion MFM scale and particularly the D1 subscore is a reliable and valid outcome applicable in longitudinal follow-up and clinical trials in the DM1 population.

Keywords Motor Function Measure; Myotonic Dystrophy type 1; Motor evaluation; Clinical trial

Disclosure of interest The authors have not supplied their declaration of competing interest.


CO020
Functional improvement with a rehabilitation programme in dermatomyositis an polymyositis: Results of a randomized controlled trial
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Objective To evaluate the medium-term functional impact and effect on quality of life of a standardized rehabilitation programme in patients with polymyositis and dermatomyositis.

Materials/patients and methods Design: a multicentre, randomized, controlled trial.
- Setting: four university hospitals in France.
- Participants: 20 patients suffering from polymyositis and dermatomyositis.
- Interventions: the intervention group participated in a four-week standardized, hospital-based rehabilitation programme and then a personalized, self-managed, home-based rehabilitation programme. The control group received Physiotherapy on an outpatient basis. The study participants were evaluated at inclusion, at the end of the rehabilitation programme (1 month) and then at 6 and 12 months.
- Main outcome measure(s): the primary efficacy criterion was the health assessment questionnaire disability index (HAQ-DI), and
the secondary criteria were quality of life (according to the SF36 questionnaire), muscle performance (isokinetic strength, the motor function measure, and the Kendall manual muscle test (MMT), gait, pain, fatigue and biomarkers of tolerance and disease activity. Results At 12 months, the mean ± standard deviation HAQ-DI was significantly lower in the intervention group than in the control group (0.64 ± 0.53 vs. 1.36 ± 1.02, respectively; \( P = 0.026 \)). The intervention group also had better scores than the control group for some quality of life dimensions (SF36 General Health: 53.44 ± 8.73 vs. 36.57 ± 22.10, respectively; \( P = 0.038 \); SF36 role physical (63.89 ± 43.50 vs. 17.86 ± 37.40, respectively; \( P = 0.023 \)), the Kendall MMT score (85.89 ± 16.11 vs. 65.22 ± 31.50, respectively; \( P < 0.05 \)) and pain levels (5.0 ± 6.61 vs. 33.38 ± 36.68, respectively; \( P = 0.04 \)) at 12 months. Lastly, the programme was well tolerated by all the participants.

Discussion/Conclusion In patients suffering from polymyositis and dermatomyositis, the combination of a four-week standardized rehabilitation programme and a personalized, home-based, self-managed rehabilitation programme was well tolerated and had a positive medium-term functional impact. Keywords Rehabilitation programme; Dermatomyositis; Polymyositis; Functional assessment; Quality of life. Disclosure of interest The authors declare that they have no competing interest.

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CO021

2005–2015: Ten years clinical experience in treating DMD patients by corticosteroids in Lyon

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Objective Since 2005, in France, corticosteroid therapy is now widely used in Duchenne muscular dystrophy (DMD). This treatment has changed our practice of pediatric rehabilitation teams. We describe here our 10-year clinical experience in treating DMD patients by CS according to international guidelines i.e. prednisone 0.75 mg/kg/day started from the plateau of motor function. Materials/patients and methods We conducted a prospective study. Information was given on the expected effects and side effects. A diet was prescribed. At baseline, a cardiac ultrasound, an ophthalmologic examination, a bone density test, a physical and biological examination to ensure the absence of against indication, check the anti-varicella immunization. The motor function measure were performed each 6 months with a medical examination and blood sample to verify the tolerance and adapt the treatment function to weight and side effects.

Results During this 10-year period, CS was proposed to 51 patients and 48 patients were finally treated up to 10.5 years. The age of onset of CS varies from 6 years to 20 years between 2005 and 2010, from 5 to 8 years after 2010. Premature stops were observed for 14 patients: 9 in the first 2 years, 5 after 2 years for side effects or inefficacy. No acute adrenal insufficiency was observed. During the first 2 years of treatment, MFM D1 score (standing position and transfers) remained stable. Then a slow decrease was observed after 2 years to be null at M72. MFM D3 score (distal motor function) is more preserved than D2 (axial and proximal motor function) and remained stable during the follow up for the great majority of patients. Eleven patients started corticosteroids after losing the ability to walk (mean age 9.1 ± 1.6 years), 7/11 had spinal fusion. Thirty-seven ambulant patients started, in this group, the mean age of losing ambulation was 11.3 ± 2.2 years and no patient had spinal surgery.

Discussion/Conclusion Increased appetite, irritability, cushingoid facies are common but well tolerated by children in view of efficacy. Dose adjustments were discussed case by case if accelerated weight gain, sluggish growth in stature and behavioral disorders.

Keywords Duchenne muscular dystrophy; Corticosteroid; MFM; Prednisone

Disclosure of interest The authors have not supplied their declaration of competing interest.

Further readings Neuromuscular Diseases.

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CO022

Seated postural in wheelchair in NMD

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Objective To study seated postural control in neuromuscular disorder.

Materials/patients and methods We conducted a retrospective observational cohort study of 130 neuromuscular adult patients having a positioning wheelchair consultation in Foundation of Garches. The assessment is done with the seated postural control measure for adults.

Results Most of the patients had severe intensity illness, only 10% were walking and 29% were with tracheostomial ventilation. The reasons of consultation where: positioning, choice or change of wheelchair, pain and prevention (rarely). The own wheelchair’s patient was powered wheelchair with seat adapted to the person type 2 in only 91 cases. 109 patients (84%) experienced pain in their wheelchair. In Duchenne muscular dystrophy patients, 44 (88%) were painful in their wheelchair. The topography of pain is frequently ischiatic. 19 patients (14%) had a pressure ulcer. All the patients examined have deformities. In Duchenne muscular dystrophy, pelvic obliquity and trunc tilt are frequently observed; in FSHD pelvic anterior tilt is frequent, and in steinert myotonic posterior tilt is frequent.

Discussion/Conclusion In order to improve the quality of life of this population, a study about the posture in wheelchair is therefore essential, most precociously.

Keywords Neuromuscular disorder; Wheelchair; Seating postural control; Pain; Pressure ulcer

Disclosure of interest The author declares that she has no competing interest.

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CO023

Muscle activations during gait in children with Duchenne muscular dystrophy

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