Title: Validity and Reliability of a New Clinical Myotonia Rating Scale for Non-Dystrophic Myotonia

Authors:

Savine Vicart[a], Jérôme Franques[b], Françoise Bouhour[c], Armelle Magot[d], Yann Péréon[d], Sabrina Sacconi[e], Aleksandra Nadaj-Pakleza[f,], Anthony Behin[g], Christine Payan[h,1], Bertrand Fontaine[a]

Affiliations:

a. Assistance Publique-Hôpitaux de Paris, Sorbonne Université, INSERM, Service of Neuro-Myology, Muscle Channelopathies Reference Center and UMR 974, Institute of Myology, University Hospital Pitié-Salpêtrière, Paris, France

b. Assistance Publique-Hôpitaux de Marseille, Department of Neurology and Neuromuscular Diseases, La Timone Hospital, Marseille, France

c. Electroneuromyography and Neuromuscular Disorders Department, Hospices Civils de Lyon, University Hospital of Lyon, France

d. Reference Centre for Neuromuscular disorders AOC, University Hospital, Hôtel-Dieu, Nantes, France

e. Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France

f. Reference Centre for Neuromuscular disorders NEIdF, Neurology Department, University Hospital of Strasbourg, Strasbourg, France

g. Assistance Publique-Hôpitaux de Paris, Service of Neuro-Myology, Reference Centre for Neuromuscular disorders NEIdF, University Hospital Pitié-Salpêtrière, Paris, France

h. Assistance Publique-Hôpitaux de Paris, Sorbonne Université, Pharmacology Department, University Hospital Pitié-Salpêtrière, Paris, France

[1] Present address: Department of Biostatistics, Clinical Epidemiology, Public Health and Innovation in Methodology (BESPIM), Nîmes University Hospital, Nîmes, France.

Abstract topic: Muscle and neuromuscular junction disorder

Introduction: The severity of myotonia is difficult to assess without the use of a standardized and validated tool.

Methods: The Clinical Myotonia Rating Scale (CMRS) was evaluated in Myotonia Congenita (MC) and Paramyotonia Congenita (PMC) patients during the randomised cross-over double-blind mexiletine vs placebo MYOMEX trial. The CMRS comprises two consecutive sections: a myotonia severity scale rated on the patient's clinical exam and a disability scale rated on the patient's opinion on daily activities. The CMRS was assessed by two different investigators at baseline and by one of them at the end of each treatment period. Interrater reliability was estimated by weighted Kappa coefficients. Intraclass correlation coefficients (ICC) were calculated for the global scores (GS). Bland & Altman methods were also used. Spearman correlation coefficients were estimated for correlations with the stiffness score using visual analogue scale (VAS) and the Individualized Neuromuscular Quality of Life (INQoL) self-questionnaire.

Results: 13 MC patients and 12 PMC patients were evaluated at six centres. Kappa ranged between -0.02 and 0.82. The highest interrater agreement was for eyelid blinking frequency and respiratory muscle intensity items (0.73 95%confidence interval [0.54;0.91] and 0.72 [0.45;0.98] respectively) as well as for hygiene and getting dressed (0.82 [0.59;1.00] and 0.73 [0.45;0.98] respectively). The ICC severity score was 0.54 and the ICC disability score - 0.65. The severity GS was strongly correlated with both the VAS (0.70, $p \leq 0.001$) and the INQoL (0.67, $p \leq 0.001$).

Conclusion: The CMRS is a promising scale and requires further validation in myotonic disorders.

Disclosures

Savine Vicart, Yann Pereon, Sabrina Sacconi and Bertrand Fontaine have received consulting fees from Lupin for other initiatives.