

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

S Vicart^a, J Franques^b, F Bouhour^c, A Magot^d, Y Péréon^d, S Sacconi^e, A Nadaj-Pakleza^f, A Behin^g, C Payan^h, B Fontaine^a

^aSorbonne University, INSERM, Muscle Channelopathies Reference Center and Institute of Myology, University Hospital Pitié-Salpêtrière, Paris, France; ^bLa Timone Hospital, Marseille, France; ^cUniversity Hospital of Lyon, Lyon, France; ^dReference Centre for Neuromuscular disorders AOC, University Hospital of Nantes, Nantes, France; ^eUniversity Hospital of Nice, Nice, France; ^fReference Centre for Neuromuscular Disorders NEIdF, University Hospital of Strasbourg, Strasbourg, France; ^gReference Centre for Neuromuscular Disorders NEIdF, University Hospital Pitié-Salpêtrière, Paris, France; ^hAssistance Publique-Hôpitaux de Paris, University Hospital Pitié-Salpêtrière, Paris, France

Introduction

- The severity of myotonia is difficult to assess without standardized and validated tools.
- This study assessed the validity and reliability of the Clinical Myotonia Rating Scale (CMRS), a novel measure for the impact of myotonia.

Methods

CMRS components

Myotonia severity scale

Body area	Severity (S)	Frequency (F)	Formula (score range)
Eyelids	0 (none) to 4 (severe, permanent)	0 (none) to 4 (every day)	$S \times F \times 0.5$ (0 to 8)
Eyes	0 (normal) to 4 (severe, diplopia)	0 (none) to 4 (every day)	$S \times F \times 0.5$ (0 to 8)
Chewing and swallowing	0 (normal) to 4 (unable to chew, choking)	0 (none) to 4 (every day)	$S \times F \times 0.5$ (0 to 8)
Upper limbs	0 (none) to 4 (severe, permanent)	0 (none) to 4 (every day)	$S \times F$ [left + right] (0 to 32)
Lower limbs	0 (none) to 4 (severe, permanent)	0 (none) to 4 (every day)	$S \times F$ [left + right] (0 to 32)
Respiratory muscles	0 (no difficulty) to 4 (permanent dyspnea)	0 (none) to 4 (every day)	$S \times F$ (0 to 16)
		Total	104

Disability scale

Disability scale	Scoring	Score range
Talking	0 (normal) to 4 (incomprehensible)	0 to 4
Writing	0 (normal) to 4 (unable to handle pen)	0 to 4
Eating	0 (normal) to 3 (dependent on others)	0 to 3
Hygiene	0 (normal) to 4 (requires 100% help)	0 to 4
Dressing	0 (normal) to 4 (requires 100% help)	0 to 4
Walking	0 (normal) to 4 (wheelchair)	0 to 4
Stair climbing	0 (normal) to 4 (impossible)	0 to 4
	Total	27

Validity and reliability testing in the MYOMEX trial

- The CMRS was evaluated in patients with myotonia congenita (MC) and paramyotonia congenita (PMC) during the randomized, crossover, double-blind MYOMEX trial of mexiletine versus placebo.¹
 - Two investigators used the CMRS to assess myotonia severity at baseline; this was repeated by one of them at the end of each treatment period in MYOMEX.
- Interrater reliability was estimated by weighted Kappa coefficients.
- Intraclass correlation coefficients (ICC) were calculated for global scores (GS). Bland-Altman methods were also used (data not shown).
- Spearman correlation coefficients were estimated for correlations with the stiffness score using a visual analogue scale (VAS) and the Individualized Neuromuscular Quality of Life (INQoL) self-questionnaire.

Results

- Patients with MC (n=13) and PMC (n=12) were evaluated at six centres in France.
- Kappa** ranged from -0.02 to 0.82, with **most showing fair/good or excellent** interrater reliability.²
 - Severity:** Highest interrater agreement for frequency of eyelid blinking (0.73) and severity of respiratory muscle intensity (0.72).
 - Disability:** Highest interrater agreement for hygiene (0.82) and getting dressed (0.73).
- Intra-class correlation coefficient** severity (0.54) and disability scores (0.65) indicated moderate interrater reliability.³

Weighted kappa coefficient (95% CI)

Severity score

Frequency

Eyelids	0.73 (0.54, 0.91)
Eyes	0.62 (0.40, 0.84)
Chewing/swallowing	0.67 (0.44, 0.90)
Right + left UL	0.40 (-0.08, 0.88)
Right + left LL	0.58 (0.19, 0.97)
Respiratory muscles	0.61 (0.33, 0.89)

Severity

Eyelids	0.65 (0.46, 0.84)
Eyes	0.44 (0.18, 0.71)
Chewing/swallowing	0.60 (0.38, 0.81)
Right UL	-0.02 (-0.07, 0.03)
Left UL	0.35 (-0.04, 0.73)
Right + left LL	0.23 (-0.02, 0.48)
Respiratory muscles	0.72 (0.45, 0.98)

Disability score

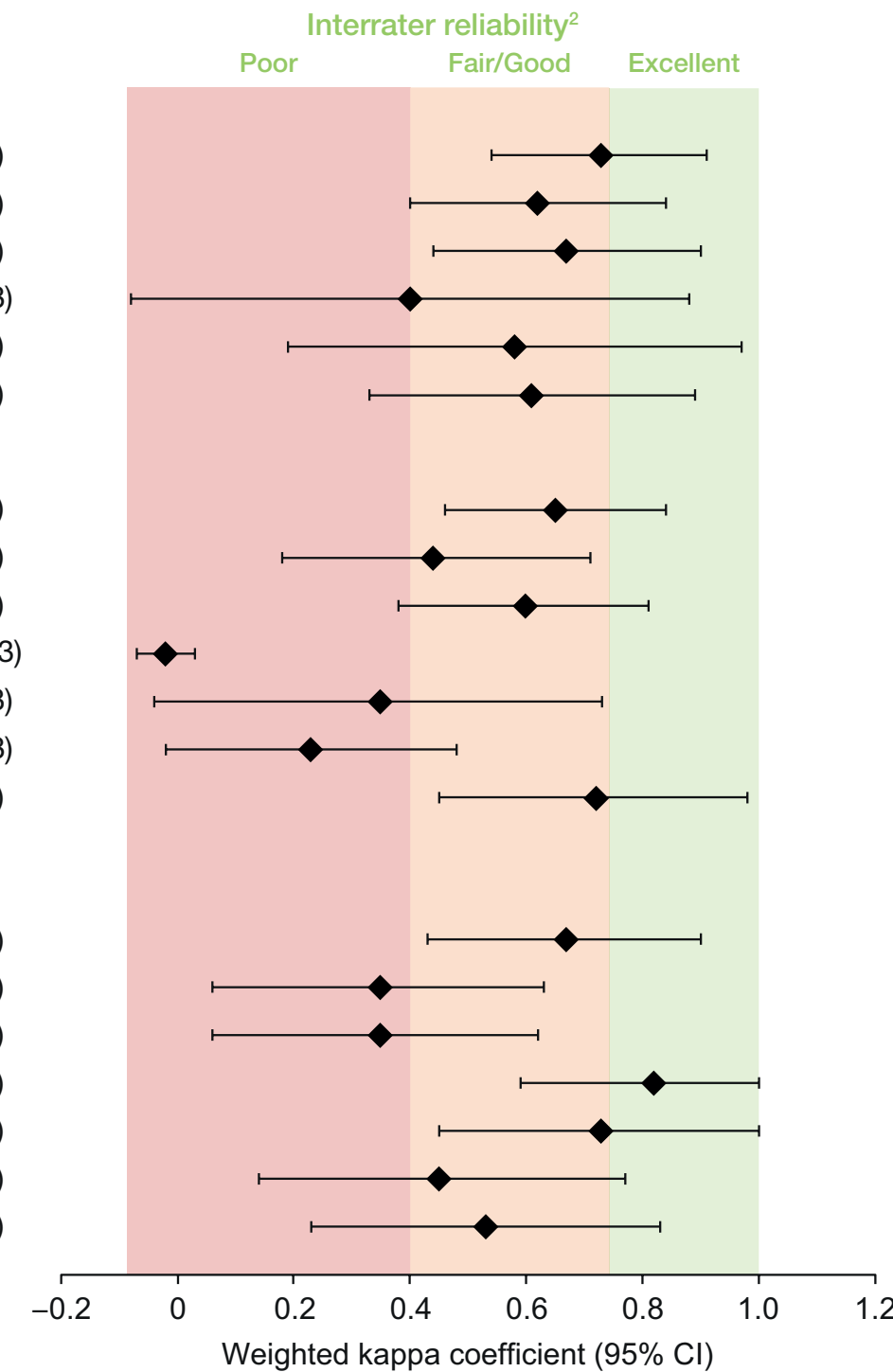
Speech	0.67 (0.43, 0.90)
Writing	0.35 (0.06, 0.63)
Eating	0.35 (0.06, 0.62)
Hygiene	0.82 (0.59, 1.00)
Dressing	0.73 (0.45, 1.00)
Walking	0.45 (0.14, 0.77)
Stair climbing	0.53 (0.23, 0.83)

Severity score

0.54

Disability score

0.65



CI, confidence interval; ICC, intra-class correlation coefficient; LL, lower limb; UL, upper limb

- Severity global score** strongly correlated with both **VAS** (0.70; $p \leq 0.001$) and **INQoL** (0.67; $p \leq 0.001$) scores.

	CMRS	INQoL	VAS
	Severity global score	Disability global score	Quality of life
Severity global score	1	0.73 ($p \leq 0.001$)	0.67 ($p \leq 0.001$)
Disability global score	0.73 ($p \leq 0.001$)	1	0.47 ($p \leq 0.001$)
			Stiffness score
			0.70 ($p \leq 0.001$)
			0.69 ($p \leq 0.001$)

CMRS, Clinical Myotonia Rating Scale; INQoL, Individualized Neuromuscular Quality of Life; VAS, visual analogue scale

Conclusions

- The CMRS is a promising scale for assessing the severity and impact of myotonia in patients with NDM.
- In this small exploratory analysis, the CMRS scoring system demonstrated moderate interrater reliability.
- The CMRS will undergo further validation in study populations with myotonic disorder.

References

- Vicart S, et al. Neuromuscul Disord 2021;31:1124-1135.
- Fleiss JL. Statistical methods for rates and proportions (2nd ed). New York: John Wiley, 1981.
- Koo TK, et al. J Chiropractic Med 2016;15:155-163.

Acknowledgement

Professional medical writing and editorial assistance were provided by Linda Edmondson, Bognor Regis, UK, funded by Lupin Neurosciences.

Disclosures

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

