The Revised Hammersmith Scale for Spinal Muscular Atrophy: Reliability, validity and results from a large international pilot

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BACKGROUND:

• Robust outcome measures are essential to assess disease progression, stability, improvement and to measure treatment efficacy.
• Outcome measures currently used in SMA 2 and 3 capture progression of disease but have limitations at the two extremes of clinical severity, and require further validation regarding their psychometric properties.
• An international collaboration between SMA REACH UK, Italian SMA Network and the PNCIRN SMA Network (USA) have been working over the last two years to address this by developing the Revised Hammersmith Scale for Spinal Muscular Atrophy (RHS).

AIM:

• To develop a psychometrically robust outcome measure for the assessment of gross motor functional abilities in type 2 and 3 SMA

METHODS:

RHS SCALE DEVELOPMENT:

• Concept of Interest & Context of Use:
  - Evaluative tool assessing gross motor function across the spectrum of abilities seen in SMA type 2 and 3.
• Underpinning Construct:
  - Reflective conceptual model of motor performance in SMA.
  - Capacity to demonstrate improvement not seen previously.
• Clinical Content Validity:
  - Determined by expert panel of Physiotherapists and Clinicians representing the three international networks.
  - Using the HFMS as a foundation an iterative process was undertaken to develop the RHS, figure 1.

RHS PILOT TESTING:

• International Pilot:
  - Participants: Children and adults with SMA.
  - RHS version 17.03.2015.
  - March to September 2015.
  - 3 national networks, 7 sites.
• Analysis:
  - Psychometric properties via the Rasch model, Rumm2030 software.
  - Additional validity/reliability analysis using SPSS version 22.

RHS INTER & INTRA-RATER RELIABILITY:

• Reliability Testing Protocol:
  - Participants: UK North Star Network Physiotherapists trained on the RHS.
  - Inter-rater testing: Secure online survey, two videos of RHS assessments, one SMA 2 and one SMA 3 patient, scored by the participants.
  - Intra-rater testing: Two weeks following inter-rater testing same videos rescored via online survey.
• Analysis:
  - Inter-rater reliability: Type 2 ICC for absolute agreement and 95% confidence intervals.
  - Intra-rater reliability: Type 2 ICC for absolute agreement and Bland Altman (BA) Limits of Agreement (LOA) and plots.

RESULTS:

RHS SCALE CONTENT:

• 36 items: Revised versions of items from the HFMS (31 items), NSAA (16 items), WHO motor milestones (2 items) and CHOP INTEND (1 item).
• Scoring: 33 items with ordinal scoring 0, 1, 2 (0 denotes least ability, 2 highest ability), 3 with binary scores 0, 1 (0 unable, 1 able). Maximum achievable score = 69.
• Ordered to limit position change.
• 2 timed tests: 10 metre run, rise from floor.
• WHO motor milestones completed alongside assessment.
• Combined RHS & HFMS proforma, performed jointly without increasing assessment time.

RHS PILOT:

• Subjects: n = 140, SMA types 1, 2 and 3, aged from 1 to 51 years, table 1.
• Floor/Ceiling Effect: Floor effect n = 1, no ceiling effect, highest score achieved 68 (n = 1).
• Psychometric Analysis: 3 invalid items, 1 extreme score, 137 assessments analysed.
  - Fit of construct: Good, no items outside ±2.5, one item (side lying) with significant v² probability (p = 0.001).
  - Reliability: Good, Person Separation Index (PSI) 0.98.
  - Items: Logical & hierarchical scores in 27/36 items, figure 3b.
  - Targeting: Excellent, minimal ceiling, weaker patients had fewest items measuring their ability, figure 3a.
  - Dependency: Noted between items assessing left & right, PSI not affected by their removal.
• Groups Validity: SMA type, current functional ability according to the WHO motor milestones and ambulation status, all p > 0.001, table 1, figures 4 a & b.
• Construct & Concurrent Validity: Strong positive correlation with WHO motor milestones r = 0.889, p < 0.001.

RHS INTER & INTRA-RATER RELIABILITY:

• Inter-rater reliability: n = 22, type 2 ICC 0.997 (0.984, 1.00), 97.7% values within ±2.
• Intra-rater reliability: n = 21, type 2 ICC 0.996 (0.985,1.00), 97.1% values within ±2.

CONCLUSION:

• The RHS is able to assess a broad range of physical abilities seen in SMA 2 and 3.
• Initial findings demonstrate the RHS is psychometrically robust, with excellent inter and intra-rater reliability. It has comprehensively addressed the shortcomings observed in the original scale.
• In very weak patients the RHS should be used in conjunction with the CHOP INTEND for infants, Revised Upper Limb Module (RULM) or patient reported outcome measures.
• Work is underway to investigate the clinical meaningfulness of the nine items with disordered thresholds to identify their relevance in specific sub-populations, in detecting longitudinal changes and the effect of Salbutamol.
• Change over time is currently being investigated.