**Revised Hammersmith Scale for Spinal Muscular Atrophy (RHS)**

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**BACKGROUND:**

- Outcome measures which meet standards set by modern psychometric analysis as well as classical measures of reliability are viewed more favourably as robust tools of choice for use in clinical trials.
- Recent psychometric analysis identified shortcomings in the clinically reported outcome measures currently used to assess motor function in SMA1.
- An international collaboration between SMA REACH UK, the Italian SMA Network and the PNCB SMA network (USA) have been working to address the shortcomings observed in functional outcome measures currently used for SMA type 2 and 3 to ensure that functional scales used in SMA are robust and fit for purpose1.

**AIM:**

To develop a robust functional clinician rated outcome measure to be used clinically and in clinical trials for use in ambulant and non-ambulant SMA type 2 and 3.

**METHODS:**

- Physiotherapists and Clinicians from SMA REACH UK, the Italian SMA Network and the PNCB SMA USA undertook an iterative process to revise the Hammersmith Functional Motor Scale Expanded (HMSE) using item response theory via the Rasch Measurement Method, expert panels and three international pilots.

  - **Scale development:**
    - The expert group meticulously discussed each item of the HMSE, scoring criteria, psychometric properties and the experience of use in clinical trials. This process highlighted item repetition, the need to adjust/define criteria and additional items.
    - Two draft revised scales were piloted internationally: Exploratory HMSE piloted January – May 2014 (n = 52), Revised Hammersmith Scale (Draft) June to December 2014 (n=70), and the above process repeated until agreement was achieved on the final version of the scale, the Revised Hammersmith Scale for SMA (RHS), in March 2015.
    - The RHS consists of 36 items to measure weak type 2 SMA through to strong type 3 SMA. Each item is graded on an ordinal scale of 0, 1, 2 except 3 items which are scored 0, 1. It incorporates items from the North Star Ambulatory Assessment (NSAA) and additional WHO developmental milestones.
    - The RHS was piloted in 3 international networks across 7 sites from March – September 2015.
    - Psychometric properties of the scale were analysed using Rumm2030 software, additional scale analysis was conducted using SPSS version 22.

**RESULTS:**

- **Subjects:** n = 140, please refer to table 3 for more detail on subject demographics. Rasch analysis 3 invalid results, 2 extreme scores

  - **Psychometric properties – Item response theory utilising RASCH Measurement Method:**
    - Very good fit of all 36 items to the construct of motor performance in SMA, table 1. No items with a fit residual outside of ±2.5, and only one item had a significant χ2 probability (p = 0.001, table 2).
    - Good reliability as demonstrated by a high Person Separation Index - PSI (0.97), table 1.
    - Logical and hierarchical individual item scores for 27/36 items, figure 3.
    - Targeting excellent with minimal ceiling, figure 2. Weaker non-ambulant patients had fewer items which measured their ability.
    - Dependency was noted between items which assess left and right and similar items such as rolling from prone to supine and supine to prone.

  - **Groups Validity:**
    - The RHS differentiates between clinically different groups: SMA type (p < 0.01), WHO condition (p = 0.001), ambulation status (p = 0.01) and Salbutamol use (p = 0.005), table 3 and figures 4 to 7.
    - The RHS has a strong significant positive correlation with the WHO motor milestones r = 0.860, p < 0.01.

  - **Type 3 Subgroup Analysis RHS vs RHS Timed Tests**
    - A moderate negative correlation was observed between RHS total score and timed rise from the floor (RHS item 25) r = -0.513, p = 0.061, r2 = 0.332, figure 8.
    - A very strong significant negative correlation was observed between the RHS total score and timed 10 metres (RHS item 19) r = -0.939, p = 0.00055, r2 = 0.605, figure 9.

**CONCLUSION:**

- The RHS is able to test the physical abilities of patients with type 2 and 3 SMA and has improved the psychometric properties of the original scales, the outstanding concerns for a few items will be addressed following discussion with the expert panel to simply scoring criteria.
- A floor effect is noted with the weaker type 2 patients. Since gross motor assessment becomes less pertinent in the very weak patients the RHS should be used in conjunction with a more sensitive scale such as the CHOP INTEND for infants, Revised Upper Limb Module (RULM) or patient reported outcome measures.
- The RHS is able to differentiate between clinically different SMA groups, and is significantly correlated with WHO developmental milestones thereby demonstrating both construct and concurrent validity.
- We are currently establishing additional validity and reliability properties of the scale. Future work will incorporate defining longitudinal trajectories using the RHS within different sub-groups of patients with SMA.


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