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Temporal dynamics of the Scale for the Assessment and Rating of Ataxia in Spinocerebellar ataxias.

Background

SARA scale is the reference clinical scale to assess the severity of cerebellar ataxia. In a context of upcoming therapeutics trials, a reliable clinical outcome is needed to assess the efficiency of the treatments.

Objectives

To precisely assess the temporal dynamics of SARA scale.

Methods

We analyzed data from four cohorts (EUROSCA, RISCA, CRC-SCA, SPATAX) including 1210 participants and 4092 visits. The linearity of the progression and the variability was assessed with an ordinal Bayesian mixed effect model (Leaspy). We did sample size calculations for therapeutics trials with different scenarios to improve the responsiveness of the scale.

Results

Seven of the eight different items had a non-linear progression. The speed of progression was different between most of the items with an average time for a one-point increase from 3.5 years [3.4;3.6] (median, 95% credible interval) for the fastest item to 11.4 [10.9;12.0] years. The total SARA score had a linear progression with an average time for a one-point increase of 0.95 [0.92;0.98] years. After removing the four last items and re-scaling all items from 0 to 4, variability increased and progression was slower, and thus would require larger sample size in a future therapeutic trial.

Conclusion

Despite a heterogeneous temporal dynamic at the item level, the global progression of the SARA scale was linear. Changing the initial scale deteriorates the responsiveness. This new information about the temporal dynamic of the scale should help to design outcome of future clinical trials.

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