New measures of neurological disability derived from NARCOMS registry data

E. Chamo1, I. Kister2, G.R. Cutter3

Dept. of Epidemiology, University of Alabama at Birmingham1, New York University Hospital for Joint Diseases-Multiple Sclerosis Center2, Dept. of Biostatistics, University of Alabama at Birmingham3

Background

There is an acute need for a quantitative summary outcome measure to assess global neurological disability from the perspective of MS patients. Existing measures are burdensome, clearly ordinal, and/or overemphasize physical disability over mental disability.

Objectives

To estimate (nearly) linear patient-reported scores of global neurological disability in MS by applying Item Response Theory (IRT) methods to North American Research Committee on Multiple Sclerosis (NARCOMS) data and: 2) assess relations between IRT-derived disability scores and patient characteristics

Measures

Disability measures consisted of 11 single-item (6-to-7-point) Performance Scales - PS (mobility, dexterity, spasticity, tremor/coordination, sensory, bowel-bladder, cognition, fatigue, pain, depression, vision) and the Patient-Determined Disease Steps (PDDS), which approximates steps of the Expanded Disability Status Scale (EDSS). Other patient characteristics included gender, age at assessment, race/ethnicity, disease modifying therapy, disease duration, year of assessment, and employment status.

Sample and methods

Study data: Intake surveys collected in 1998-2011. Analyses were restricted to 7,851 patients with complete PS data. The sample was randomly split into development (n=3,926) and validation (n=3,925) sets.

Methods: Item factor analysis and IRT analysis were used to evaluate the measurement structure of the 11 PS and to generate IRT scale scores of disability expressed on the standard deviation (z-score) metric. We applied three methods of IRT scale score estimation: plausible score (gold standard, laborious), Bayes expected a posteriori (EAP, most precise but biased), and summed score EAP (SSEAP, simplest, most convenient in practice but most biased). We employed linear regression to identify patient characteristics associated with each disability score and logistic regression to test the hypothesis of independent association of the scores with unemployment.

Results

Average inter-item tetrachoric correlation was 0.41, Cronbach's alpha was 0.88, and median item-total correlations was 0.70 (range, 0.57-0.77).

Factor and IRT analyses did not clearly indicate whether the measurement structure of disability was best represented by a one-dimensional model (left figure above) or by a bifactor model including a general factor of global disability and two uncorrelated auxiliary factors accounting for unexplained residual variance of "physical" and "mental" disability (right figure). Several results suggested that the factor of global disability was "strong" and the auxiliary factors "weak". For instance, 87.8% of reliable variance in PS raw summed scores was explained by the dimension of global disability and only 12.2% by the auxiliary factors ($Q_{PS}=0.82$ and $Q_{total}=0.91$).

Scores of both global and physical disability strongly discriminated among PDDS levels. The relation between mental disability and PDDS was more tenuous, possibly reflecting the lack of sensitivity of the PDDS to mental disability or low participation in NARCOMS of severely impaired patients. The method of IRT score estimation (plausible, EAP, SSEAP) had relatively little impact on scores of global disability, but more on the other two scores.

Conclusions

There are several IRT options to generate PS-based scores of global disability. All of them yield scores that have better metric properties than the raw summed score composite. The simplest methods of score derivation might prove useful in clinical practice and the most complex in research. The three dimensions of disability defined by the bifactor model have independent explanatory power, but the auxiliary dimensions are weak (poorly identified).

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