OBJECTIVES: ‘Daytime functioning’ is a widely used outcome to describe quality of life. A search was conducted of the English-language studies published between January 1950 and June 2008 using Medline. Key terms used were ‘daytime function and questionnaires’, ‘daytime functioning’, ‘daytime functioning and quality of life’. Studies that observed functional performance from adult populations were exclusively selected and of those articles, patient-reported questionnaires were identified. Validity of instruments was assessed based on published psychometric properties. RESULTS: Total of 55 articles were identified. Studied patient population had sleep disorders (including apnea) (n = 47), cancer (n = 2), restless leg syndrome (n = 2), allergic rhinitis (n = 1), Alzheimer’s disease (n = 1), osteoarthritis (n = 1), Parkinson’s disease (n = 1). Of these, 45% (n = 25) articles included a patient-reported outcome questionnaire that claimed to measure daytime functioning. The remaining articles (55% n = 30) included objective measurement to describe daytime function or used questionnaires without the supporting evidence of reliability, validity and interpretability. The three validated PRO tools used to measure changes in patients’ functioning were observed from different areas: generic disease state (i.e. health, social support and activities) (n = 4), sleep disorders (n = 4), psychiatry (n = 3) and fatigue (n = 1). CONCLUSIONS: Not all studies that purported to measure daytime functioning used validated PRO tools to measure changes in daytime functioning. The criteria of defining daytime functioning were different for different disease states. Future studies that will investigate the domain of daytime functioning, it is recommended to select appropriate validated PRO instruments that can support the intended claim.

FUNCTIONAL ASSESSMENT OF MULTIPLE SCLEROSIS: RESULTS OF A LARGE MULTINATIONAL OBSERVATION STUDY

Günther OH1, Mittenburger C1, Pozzilli C1, Oerlemans W1
1 Innoovis Berlin, Berlin, Germany, 2Innoovis Berlin, Berlin, Germany, 3University of Rome La Sapienza, Rome, Italy, 4Bayer Schering Pharma AG, Berlin, Germany

OBJECTIVES: Health-related quality of life (HRQoL) is an important outcome in multiple sclerosis (MS). As part of the measurement system “Functional Assessment of Chronic Illness Therapy (FACIT)” targeted to the management of chronic illnesses, the Functional Assessment of MS (FAMS) is a self-reported questionnaire assessing patient-reported treatment effects in MS studies. Although the FAMS is validated in MS, the link between meaningful clinical efficacy and FAMS change remains unclear. Objective was to determine anchor-based “clinical important differences” (CID) in FAMS total score (FAMS TS) and FAMS trial outcome index (FAMS TOI) using disability as measured by the Expanded Disability Status Scale (EDSS).

METHODS: 6 months data from a prospective, observational study that included patients with relapsing MS were used. All patients had been switched from another drug to interferon beta 1-b 1–3 months before inclusion. The EDSS, the FAMS-TOI were assessed at baseline and 6 months, with 1078 respondents at baseline and 1047 at follow-up. Regression analysis was used to estimate CID of FAMS change scores by three anchor categories of EDSS change (“deteriorated”, “unchanged”, “improved”). Distribution based measures (standardized effect size [SRM]) were used to quantify the strength of CID. RESULTS: CID of 6 months was 5.65 [95% confidence interval: 0.75;10.56] (FAMS TS) and 4.22 [0.02;8.42] (FAMS TOI). Patients with EDSS improved had significantly larger CID than for EDSS unchanged with SRM of 0.24 and 0.23, respectively. CONCLUSIONS: CID estimates are provided for improvement in HRQoL in patients with MS over a six-month period. The results are consistent with published CID of clinical trials ranging from 4 to 8 units in patients with cancer. The estimated CID can assist clinicians and health policy makers in evaluating significance of short-term treatment effects of medical as well as non-medical interventions (e.g. patient support programmes).