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REVIEW OF PATIENT-REPORTED OUTCOME MEASURES FOR DAYTIME FUNCTIONING

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OBJECTIVES: 'Daytime functioning' is a widely used outcome to describe quality of patients' functional performance during daytime and gaining popularity as an efficacy outcome in pharmaceutical interventions. The purpose of this study was to conduct a review of the literature and identify studies and patient-reported outcome instruments that purport to measure daytime functioning. **METHODS**: 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 and quality of life', 'daytime functioning and questionnaires', and 'daytime functioning and quality of life'. Studies that observed functional performance from adult population were exclusively selected and of those articles, patient-reported questionnaires were identified. Validity of instruments was assessed based on reported 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), coronary artery disease (n = 1), overactive bladder (n = 1), and 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 performance or used questionnaires without the supporting evidence of reliability, validity and interpretability. After excluding duplicated PRO-questionnaires, 13 tools were identified and changes in patients' functioning were observed from different areas; generic disease state (i.e. health, social support and activities survey) (n = 4), sleep disorders (n = 4), psychiatry (n = 3) and fatigue (n = 1). **CONCLUSIONS**: Not all studies that purport to measure daytime functioning used validated PRO tools to measure changes in daytime functioning. The criteria of defining daytime functioning were differed among 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.

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FACTORS DRIVING PATIENT SELECTION OF PRIMARY MEDICATION FOR MULTIPLE SCLEROSIS

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OBJECTIVES: To evaluate the factors driving selection of the primary medication for multiple sclerosis (MS) by patients. **METHODS**: Baseline data from ROBUST, which is a 12-month, US prospective, observational, open-label, single-arm, multi-center outcomes study of Interferon β-1b given every other day for relapsing forms of MSwere analyzed. At baseline, patients were asked to rate the importance of factors that are typically considered before making a decision to select the primary medication to treat MS (on a scale of 1 = not at all important through 5 = very important). Twelve factors were evaluated: how well medication works, how much medication is given per dose, convenience of taking medication, convenience of storing medication, frequency of administration, information in product decision kit, injection needle of the medication, personal research, doctor recommendation, family opinion, opinion of other MS patients and recommendation of MS advocacy groups. Mean scores on each factor were compared across patient sociodemographic characteristics, primary medication types and disease severity. **RESULTS**: At baseline, a total of 99 reported taking medication to treat their MS and thus provided responses on the items of interest. In this sample, 80.8% were females, 61.6% married, 70.4% below 50 years old and 82.8% White. Among all patients, the highest importance scores were received for how well the medications controls MS progression (Mean [95% Confidence Interval] = 4.72 [4.57–4.86]) followed by their doctor's recommendation (4.57 [4.41–4.72]), the convenience of taking the medications (3.88 [3.63–4.13]), personal research (3.81 [3.56–4.05]) and the convenience of storing the medication (3.71 [3.44–3.98]). These factors and their order of importance remained consistent across patient sociodemographic characteristics, primary medication types and disease severity. **CONCLUSIONS**: Contributing to the limited published research about factors considered by patients when selecting primary medication for their MS, this analysis found that medication effectiveness to control MS progression and doctor recommendation are the most important drivers of medication selection.

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HEALTH-RELATED QUALITY OF LIFE, EDSS AND TIMED 25-FOOT WALK IN A MULTIPLE SCLEROSIS POPULATION OF A REAL-WORLD OBSERVATIONAL OUTCOMES STUDY: BASELINE DATA FROM ROBUST

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OBJECTIVES: To explore the association of baseline health-related quality of life (HRQoL) and clinical measures of disability and motor functioning in a real-world observational study of multiple sclerosis (MS) patients. **METHODS**: The Real-World Betaseron® Outcomes Study (ROBUST) is a US, prospective, 12-month, observational, single-arm, open-label, multi-center outcomes study of interferon beta-1b given every other day for MS. A total of 226 patients were registered at 52 neurologists' sites. Clinical measures and patient outcomes were reported by physicians and patients via a web-based data capture tool. The Kurtzke Expanded Disability Status Scale (EDSS) and Timed 25-foot Walk (T25FW; in seconds) were measured by the neurologist at baseline to evaluate patient disability and motor functioning. HRQoL was measured at baseline for 191 patients via the SF-12 Health Survey. For this analysis, EDSS and T25FW were categorized into five (0, 1–2, 2.5–4, 4.5–5.5, 6) and two (< = 7, >7 sec) groups, respectively. SF-12 results were summarized by Physical Component Score (PCS-12) and Mental Component Score (MCS-12). Analysis of variance methods were used to explore the association of baseline HRQoL and clinically measured EDSS and T25FW. **RESULTS**: At higher EDSS scores (i.e., worsening disability), both physical and mental HRQoL were lower; however, this association was only statistically significant for PCS-12 (p = < .001 for PCS-12, p = 0.707 for MCS-12). For PCS-12, among adjacent EDSS groups, pairwise differences were statistically significant between EDSS = 1–2 vs. EDSS = 2.5–4 (44.0 vs. 37.2; p = .0004), suggesting a marked worsening of physical HRQoL at or above the disability threshold of EDSS = 2.5. Both PCS-12 and MCS-12 were higher for the group with better ambulation: T25FW < = 7 sec vs. T25FW >7 sec (43.5 vs. 37.0, p < 0.001 for PCS-12; 43.9 vs. 41.0, p = 0.079 for MCS-12). **CONCLUSIONS**: Physical HRQoL is associated with functional impairment assessed via the EDSS and T25FW. This suggests that the PCS-12 component of the generic HRQoL tool reflects clinical functional impairment in MS patients.

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RESPONSIVENESS AND CLINICAL IMPORTANT DIFFERENCES OF THE FUNCTIONAL ASSESSMENT OF MULTIPLE SCLEROSIS: RESULTS OF A LARGE MULTINATIONAL OBSERVATION STUDY

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OBJECTIVES: Health related quality of life (HrQoL) is an important outcome in multiple sclerosis (MS). As part of the measurement system "Functional Assesment 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 score changes 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, observation study (BetaPlus) in patients with relapsing MS were used. All patients had been switched from another drug to interferon beta-1b 1–3 months before inclusion. The EDSS, the FAMS-TS, the FAMS-TOI were assessed at baseline and 6 months, with 1078 respondents at baseline and 950 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 at 6 months was 5.65 [95% confidence interval: 0.75;10.56] (FAMS TS) and 4.22 [0.02;8.42] (FAMS TOI). All coefficients for "EDSS improved" were significantly larger 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 inline with published CID of FACIT instruments 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).

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COSTS AND HEALTH-RELATED QUALITY OF LIFE IN PATIENTS WITH GILLES DE LA TOURETTE'S SYNDROME

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OBJECTIVES: To assess the costs and the health-related quality of life (HrQoL) of patients with Gilles de la Tourette's syndrome (GTS) in Germany **METHODS**: Direct and indirect costs as well as HrQoL were evaluated in 200 patients with GTS in three outpatient departments in Germany. Economic data was provided in a patient diary over a three-months-period. Costs were analyzed from the perspective of the statutory health insurance. Indirect costs were calculated by the human capital approach. HrQoL was measured using the EuroQol instrument. Depression was assessed using the Beck Depression Inventory (BDI) and clinical symptoms with the Yale Tourette Syndrome Symptom list (YTSSL) and the Shapiro-Tourette-Syndrome-Severity-Scale (STSSS). **RESULTS**: Direct costs amounted to €635 during the evaluation period. Indirect costs totalled to €2500. Major cost components were: drug costs (€350) and in-stays (€300). Patients with GTS proved to have a worse HrQoL than a representative sample of the general German population. In GTS patients the most affected EQ-5D domains were anxiety/depression (57.1%), pain/discomfort (47.5%), and daily activities (38.4%). The mean EQ VAS score was 65.4 ± 21.9. The patients had a mean BDI score of 12.3 ± 9.9, which was considerably worse compared to a representative German sample with a mean BDI score of 6.45 ± 5.2. The mean STSSS value was 3.24 ± 1.1. There was no significant difference between genders with respect to tic severity. A significant correlation for BDI, age, YTSSL, STSSS, subjective efficacy of