OBJECTIVES: Hemophilia is a chronic disease typically diagnosed in infancy, charac-
terized by a deficiency of factor VIII or IX. Treatment for hemophilia consists of regular or episodic infusions. Taking care of a child with hemophilia (CWH) may cause burden for caregivers. We aimed to develop a first “Hemophilia associated Caregiver Burden Scale” (HEMOCABTM) assessing the burden of hemophilia for caregivers of CWH. METHODS: Questionnaire comprising 55 questions grouped in 13 domains was pilot-tested in 40 caregivers of CWH with a mean age of 39.3±2.9. The majority of CWH had hemophilia A (95%), were severely affected by hemophilia (77.5%) and 15% had inhibitors. Reliability estimation showed high internal consistency of total score with Cronbach’s α = 0.97, and for 2 summary scores ‘frequency’ with α = 0.95 and ‘burden’ α = 0.92, Cronbach’s α for the sub domains ranged from α = 0.77 to 0.93. HEMOCABTM revealed good conver-
gence validity and different correlations and factor analysis, which support validity. Discriminant validity showed significant differences in all domains of HEMOCABTM, except for school’ among caregivers of CWH with inhibitors vs. without. Type of treatment and disease severity showed some differences between groups. Based on item and scale analysis 69 items were deleted and the final HEMOCABTM consists of 59 items. CONCLUSIONS: HEMOCABTM is the first hemophilia-specific instrument for the assessment of caregiver burden and revealed good psychometric characteristics in terms of reliability and validity.

PRM83

USING THE CLINICAL SUMMARY SCORE FROM THE KANSAS CITY CARDIOMYOPATHY QUESTIONNAIRE AS AN ENDPOINT IN CLINICAL TRIALS: PSYCHOMETRIC SUPPORT

Germain4, Tiplou1, S. Phaneaux2

1, 2ERT, Pittsburgh, PA, USA, 3Novartis Pharma AG, Basel, Switzerland

OBJECTIVES: Symptoms and physical limitations can have an important impact on the day to day lives of heart failure patients. The Clinical Summary Score (CSS) of the Kansas City Cardiomyopathy Questionnaire (KCCQ) is a patient-reported outcome instrument, provides a measure of symptoms and physical limitations associated with heart failure. The primary goal of this study was to evaluate the psychometric properties of the CSS and its utility as an endpoint in clinical trials. METHODS: Data from 3 randomized, controlled clinical trials with heart failure patients were included in analysis (ALOFT, PARAMOUNT, and PARADIGM trials). Studies were examined independently, within each study, data were collapsed across treat-
ment groups. Outcomes included the KCCQ, physician-rated New York Heart Association (NYHA) classification, patient global impression of change (PGIC), and/or NY-PROBNP assay. RESULTS: Findings were similar across the 3 trials. Mean CSS scores at baseline ranged from 63-76 (on a 0-100 scale, with higher scores indica-
ting better symptoms and physical functioning). Dimensionality assessments highlighted the complex nature of the scale, with evidence for first-, second-, and third-order factors. The CSS consistently discriminated between all four NYHA clas-
sifications (all pairwise comparisons p < 0.05). Correlations with BNP and NT-proBNP levels were statistically significant, but relatively small (r = 12 - 17 respectively). The CSS was sensitive to changes in patient status over time, as indexed by changes in NYHA classification and PGIC. The responder definition – the amount of change within an individual patient that would be considered clinically meaning-
ful - was in the range of 6 to 10 points, which is higher than what has been seen in studies using the KCCQ overall score. CONCLUSIONS: Data from multiple heart failure clinical trials confirm the psychometric characteristics of the CSS. This evidence supports the use of the CSS as an endpoint in clinical trials examining heart failure treatments.

PRM84

VALIDATION OF THE DEPRESSION AND FAMILY FUNCTIONING SCALE (DFFS) 1, 2, 3, 4, 5

J. Chen1, Y. Chen2, J. Li3, D. Chen2, D. Li4, X. Qiu3, J. Hou4, J. Wang1

1University of Southern California, Los Angeles, CA, USA, 2Children’s Hospital Los Angeles, Los Angeles, CA, USA, 3Barcelona GSE, Barcelona, Spain

OBJECTIVES: We systematically investigate random utility maximization and random regret minimization modeling approaches to establish the impact of dif-
ferences (dces) on people reported any kind of loss of workforce participation due to illnesses/treatments. Average loss of productivity was significantly higher in females vs males (56% vs 13%, p <0.05) and decreased with increase in age (20%, 17%, 14%, 11% and 7% in 18-29, 30-39, 40-49, 50-64 and > 64 years old, respectively, p-value <0.05). CONCLUSIONS: HRPQ has good construct and criterion validity. Presenteeism remains higher for paid work, while absenteeism remains higher for unpaid work.

PRM85

MEASURING UPPER LIMB FUNCTION IN MULTIPLE SCLEROSIS: ENHANCING THE ABILHAND’S PERFORMANCE

Cans1, C. Kleinbouw1, 2, Marquis P., Hobar1, 3, Naesby S., Mikol D., Pelletti1, 4, Steiner D., Chen2

1Modus Outcomes LLC, New York, USA, 2Modus Outcomes LLC, London, UK, 3Plymouth University, Plymouth, Devon, UK, 4Biogen Idec, Cambridge, MA, USA

OBJECTIVES: ABILHAND is a phase 3, randomized, double-blind, placebo-controlled trial assessing whether natalizumab slows disability progression in secondary progressive multiple sclerosis (SPMS) patients. The aim of this current analysis was to use Rasch Measurement Theory (RMT) methods to evaluate the ABILHAND PRM82's effectiveness. Methods: Data from the 889 randomized patients in ABILHAND were ana-
lyzed in stage 1, RMT methods examined: scale-to-sample targeting, item fit, local dependency, and reliability. In stage 2, a post-hoc revision of the ABILHAND-56 scoring structure and conceptual groupings of items was conducted and re-
evaluated using the same RMT methods. RESULTS: Stage 1 analyses showed adequate item performance: minor item misfit (0.56%); minimal dependency (4
d pairs of items); good reliability (Person Separation Index = 0.93). However, there was ABILHAND-to-ASCEND phase mis-targeting (person range on mean: [7.5 to 11.1, item range: [7.5 to 12.7]). In stage 2, all items were re-scored on a dichotomous response scale (easy & difficult/impossible) in an attempt to improve targeting. Also, the 56 items were re-categorized into 3 computationally clean (32), function “strength” (12), “power” (12) and “endurance” (2). The revised ABILHAND-56 RMT methods: improved targeting, test-retest reliability (intra-class correlations), construct validity (correlations and factor analysis), discriminant ability (analyses of variance), and responsiveness (effect size estimates) were evaluated.

PRM86

DOES DIFFERENTIAL FRAMING OF OPT-OUT ALTERNATIVES IN DISCRETE CHOICE EXPERIMENTS (DCEs) MATTER? COMPARISON OF RANDOM UTILITY MAXIMIZATION (RUM) AND RANDOM REGRET MINIMIZATION (RRM) MODELS

S. Oh, M. Lee, T. Yoon, K. Hwang, H. Han, J. Lee

University of Southern California, Los Angeles, CA, USA, 2Children’s Hospital Los Angeles, Los Angeles, CA, USA, 3Barcelona GSE, Barcelona, Spain

OBJECTIVES: To validate the Health-Related Productivity Questionnaire (HRPQ), a new health-related productivity instrument, and estimate the US population numbers by age and gender. METHODS: An online survey was developed that con-
sisted of four components: the HRPQ, a screener to determine relevant disease conditions-related and health-state questions; validated instruments such as Work Productivity and Activity Impairment (WPAI) and EQ-5D; and socio-demo-
graphic questions. The survey was administered by a third-party company for a 6-week period. Weightings were calculated to allow extrapolation of results from the 10,000 respondents to achieve values representing the general United States population. Validation analysis included concurrent and criterion validity, construct validity, differential items, composite and item group comparisons. Methods: Mean, median, standard error, 25th and 75thpercentiles were calculated for absenteeism, presenteeism measures for employed and household work and stratification by age, gender and caregiver status. The HRPQ showed concurrent validity with WPAI (Pearson’s r=0.6, p-value<0.05). Correlations of total productivity at work and home from HRPQ with EQ-5D scale scores were small to mod-
erate, r=0.3-0.5 (p-value<0.05) and aligned with direction of the hypothesis. Several groups of people showed positive correlations, heavily loaded on one factor and absenteeism and scheduled hours items loaded on second factor. General population estimates for average percent lost produc-
tivity at work was 14%, [absenteeism=4%, presenteeism=10%] and for household activities was 28% [absenteeism=18%, presenteeism=10%]. Furthermore, 17%