was designed to assess efficacy and safety of teriparatide, a novel oral disease modifier, in RMS patients. The utility score, a measure of health-related quality of life, was calculated using the EQ-SD questionnaire, based on the patient’s responses. Using baseline data, a cross-sectional analysis was performed to identify factors associated with utility scores using a multivariate regression linear model. This model includes the following factors: expanded disability status scale (EDSS) score, type of multiple sclerosis (relapsing remitting (RMS) versus progressive relapsing (PRMS)), region, year or relapses within the past 2 years, previous MS medication, gender, time (years) since diagnosis of MS and burden of disease defined by magnetic resonance imaging. RESULTS: Three variables demonstrated a significant negative impact on utility values: the functional disability level as assessed by EDSS score (when EDSS score increases), PRMS versus RMS and Eastern European countries versus North American countries. The major influencing factor, consistent with other analyses, was the EDSS score with the following utility estimates: EDSS=0.81 (0.52), p<0.001; EDSS=5–0.237, p<0.001, EDSS=6–0.231, p<0.001; EDSS=7–0.257, p<0.001. In addition, utilities associated with PRMS versus RMS and Eastern Europe region versus North American were respectively -0.073 (p=0.0028) and -0.036 (p=0.036). CONCLUSION: In RMS patients, these results confirm the major impact of functional disability on patients’ utility. These analyses also provided disutility estimates per EDSS score, utilities for PRMS versus RMS and for Eastern Europe versus North American region. The later probably reflecting cultural differences in health status perception.

PND39 HEREDITARY ANGIOEDEMA HEALTH STATE UTILITY VALUATION STUDY FROM THE PERSPECTIVE OF A REPRESENTATIVE SAMPLE OF THE AUSTRALIAN GENERAL PUBLIC
The authors were not identified.

OBJECTIVES: To examine resource utilization associated with the use of Rasagiline or Selegiline,

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OBJECTIVES: To examine resource utilization associated with the use of Rasagiline or Selegiline, two commonly prescribed MAO-B inhibitors for the treatment of Parkinson’s Disease (PD).

METHODS: Data for this retrospective study were obtained from the US 13 LabPlus database over the time period from January 1, 2006 through December 30, 2010. Patients were included in the analysis if they were prescribed Rasagline or Selegiline with first such date identified as the index date, were diagnosed with PD (ICD-9 code 333.0), and had continuous insurance coverage from 6 months prior through 3 months post index date. Analyses are presented descriptively in terms of mean and standard deviation.

RESULTS: There were 1242 individuals included in the study - 926 initiated on Rasagline versus 316 on Selegiline. Patients initiated on Rasagline compared to those initiated on Selegiline, were significantly younger (63.2 years vs. 65.4 years, P=0.0020). Patients initiated on Rasagline were significantly less likely to be diagnosed with chest pain (16.41% vs. 21.52%; P=0.0402) or headaches (4.97% vs. 9.49%; P=0.0037). Patients who initiated on Rasagline were significantly more likely to be followed for 6 months post treatment initiation (90.68% vs. 77.78%; P=0.0015). Compared with Selegiline use, initiation on Rasagline was associated with significantly fewer inpatient visits (1.58 vs. 2.94; P=0.0236) and significantly shorter hospital length of stay (4.71 days vs. 8.78 days; P=0.0216). CONCLUSIONS: Results from this retrospective study indicate that patients initiated on Rasagline were less likely to experience side effects of chest pain or headaches. In addition, these patients were more likely to have a greater resource utilization due to the number and significantly longer lengths of hospitalizations.

PND40 A STUDY TO ESTIMATE UTILITY VALUES FOR DIFFERENT LEVELS OF SEVERITY OF MIGRAINE PAIN

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OBJECTIVES: Health utility values are the metric preferred by health care decision makers to examine the relative value of various treatments to treat migraine, including those that reduce the severity of migraine. This cross-sectional, observational study aimed to estimate utility values for different levels of migraine pain severity. METHODS: Participants, between 18 and 80 years of age, who were diagnosed with migraine, completed the EQ-SD™ to evaluate their health status for the past week. T-tests were used to compare mean utility values between the levels of severity to evaluate whether there were significant differences in mean utility scores by migraine severity; Wilcoxon signed rank test was also performed. RESULTS: Utility scores for each health state were significantly different from perfect health (p<0.0001) and one another (p<0.0001). As severity worsened, utility decreased and the lowest mean utility, 0.20 (95% confidence interval [CI]: 0.27 − 0.13), was for severe migraine pain. Compared to current health (without migraine), utility decrements were 0.21, 0.34, and 1.07 for mild, moderate, and severe migraine pain states respectively. The smallest difference in mean utility scores was between mild and moderate migraine pain (0.13) and the largest difference in mean utility scores was between current health (without migraine) and severe migraine pain (1.07). CONCLUSIONS: Migraine pain severity was associated with significantly lower utility compared with perfect health, with higher levels of pain severity associated with lower utility. Severe migraine pain was considered a health state worse than death. Our results can be used in cost-utility models examining the relative economic value of therapeutic strategies for migraine in the UK.

PND41 PREDICTING EQ-SD UTILITY SCORES FROM THE HUNTINGTON QUALITY OF LIFE INSTRUMENT (H-QOL-I)

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OBJECTIVES: H-Qol-I is a quality-of-life indicator specific to the Huntington’s Disease (HD) (1) and, with 3 dimensions: motor function, psychology and socializing. It has been validated in several countries (France, Italy, Poland, Germany and United States). H-Qol-I can be used in cost-utility models examining the relative economic value of therapeutic strategies for HD.
squares (OLS) regression and Tobit regression, with utility score as independent variable, and ordered logistic regression for each item of EQ-5D. Model performance was assessed by comparing predicted and observed mean EQ-5D scores in the validation set, the unadjusted R-squared and the root mean squared error (RMSE). RESULTS: The OLS regression had the best predictive performance with R-squared equal to 0.60 and RMSE equal to 0.25. The linear regression model accurately predicted changes in EQ-5D score in the validation set (predicted score 0.52 versus observed score 0.30). RMSE values of 0.26 and 0.31 were obtained with Tobit model and ordered logistic model. Items with the greatest contribution to variance in the OLS model were ‘difficulty of tie the laces of my shoes’ (p < 0.001), ‘difficulty to drink without spilling’ (p = 0.0156) and ‘difficulty to make precise movements’ (p = 0.0165). CONCLUSIONS: EQ-5D utility scores can be reasonably predicted from the H-Qol-I, although item wordings are not directly related. The model based on OLS regression provides the best fit. Functioning items contributed the model to utility predictions.

PND43 IMPACT OF RELAPSES LEADING TO HOSPITALISATION ON HEALTH-RELATED QUALITY OF LIFE, FATIGUE AND HEALTH CARE RESOURCE UTILISATION IN A POPULATION WITH A RELAPSING FORM OF MULTIPLE SCLEROSIS (RMS) USING DATA FROM TEMSO A TEFURFLONIDOMIDE PIVOTAL PHASE III TRIAL O’Connor P1, Goldberg L2, Bege-Lo Bagoune C2, Dave Pouletty C2
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OBJECTIVES: In patients with RMS, assess the impact of relapse(s) leading to hospitalisation on Health-Related Quality of Life (HR-QoL), fatigue and Health Care resource utilisation. METHODS: TEMSO (N=1088) was designed to assess efficacy and safety of tefurflonidomide, a novel oral disease modifier, in RMS patients. Patients with no relapse, patients with relapse(s) not leading to hospitalisation and patients with relapse(s) leading to hospitalisation were identified. Patients with reported outcomes (PROs) were assessed, utility (EQ5D), PCS and MCS (Physical and Mental Health Component Summaries) scores of the SF-36, fatigue (FIS-total score). Also, Emergency Medical Facility Visits (EMFV, a visit to a medical facility/hospital for emergency care not resulting in an admission) was tracked. Changes from Baseline for PROs and annual EMFV rate were analysed for a two-year period. RESULTS: Change from baseline (CBF) in utility in patients with no relapse was +0.034, CBF in utility in patients with relapse(s) not leading to hospitalisation was +0.075 (p<0.001, p2 ns), and CBF in utility in patients with relapse(s) leading to hospitalisation was +0.057 (p<0.001, p2 ns). Similar results were seen for PCS of +1.0, -1.0 (p<0.01) and +3.1p<0.001, p2=0.05 respectively and for MCS were respectively +1.8, -1.0 (p<0.01) and -2.7 (p<0.01, p2 ns). This same trend was observed with FIS total score, CBF was respectively -3.0, +1.4 (p<0.01) and +10.3 (p1<0.01, p2 ns). The mean annual EMFV rate in patients with no relapse was 0.5 and in patients with relapse not leading to hospitalisation was 0.4 (p1 ns). This rate was increased to 1.2 (p1<0.05, p2<0.001) in patients with relapse leading to hospitalisation. CONCLUSIONS: In TEMSO, patients with relapse leading to hospitalisation comparatively have worsening in HR-Qol (EQ-5D, SF-36), fatigue and have a higher number of EMFV. p1: versus patients with no relapse, p2: versus patients with relapse not leading to hospitalisation.

PND44 VALIDATION OF THE SELF ASSESSMENT OF TREATMENT (SAT) QUESTIONNAIRE: CULTURAL VALIDATION IN GERMANY, POLAND AND USA Wykrzak KW1, Thompson CJ2, Holmstrom S3, Wiklund P4
OBJECTIVES: The original SAT is a five-item questionnaire developed to assess treatment benefits associated with application of QUTENZA™, a novel high-dose capsicain patch, in clinical trials among patients with neuropathic pain. The objective of this study was to evaluate the item performance and to explore the underlying constructs. Reliability and validity were also examined. RESULTS: Pooled data from 698 patients (21-91 years) completing SAT after 12 weeks of treatment were analysed. From descriptive statistics, EFA and CFA results, a one-factor model combining 4 of the 5 items emerged as the optimal solution, which explained variance. The internal consistency reliability was high (Cronbach’s alpha = 0.87). Construct validity was demonstrated by moderate to high correlations with change in the NPRS (0.55 pain now and -0.64 average pain), BPI (-0.59 worst pain, -0.35 activity limitation), SF 36v2 pain subscale (0.43) and GPC (0.64). The discriminant validity was tested through patient change groups using the GPC; mean SAT scores were 1.7 in patients who were very much improved versus -1.0 in patients who were much worse/very much worse. CONCLUSIONS: Preliminary analyses indicate that the measurement properties of the four-item version of SAT are valid and reliable for patients self assessment of treatment with QUTENZA™ among patients with neuropathic pain. The item performance suggests that the questionnaire could further improve with additional patient input to clarify some of the questions as well as revising the response options and recall period to better reflect treatment benefits during the course of a trial.

PND45 VALIDATION OF THE FABRY OUTCOME FUNCTION (FOS) PAEDIATRIC HEALTH AND PAIN QUESTIONNAIRE Bailey M1, Wiklund P2, Stull DE3, Chen WH4, Ramasamy U5, Whybra C6, Kalkun G6, Ping A7, Parisi R8, Kohrbach M9, Beck M9
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OBJECTIVES: The original 40-question Fabry-specific Paediatric Health and Pain Questionnaire (FPHQ) was developed to understand and assess the symptoms in Fabry Disease (FD) patients as no validated instrument existed. The objective of this study was to evaluate the psychometric properties of the FPHQ. METHODS: FPHQ data were collected from the FOS, a registry sponsored by Shire HGT for patients with FD who were treatment naive or receiving enzyme replacement therapy with agalsidase alfa. Descriptive statistics and exploratory factor analysis were conducted to assess the item performance and to explore the underlying constructs. Reliability, validity, and responsiveness were also examined. RESULTS: Eighty-seven children (aged 4-18 years) from 8 different countries completed the questionnaire. From descriptive statistics and EFA, 23 items in three subscales emerged: Pain associated with heat or exertion; pain associated with cold; abdominal pain and fatigue. Internal consistency reliability for all three subscales was good (Cronbach alpha = 0.84) and high for all age groups (4-7, 8-12, 13-18 years). Test-retest reliability was high for all three subscales (intraclass correlation coefficient = 0.74). Construct validity was demonstrated by moderate correlation with the Fabry Pain Inventory (FPI), KINDL, and EQ-SD. Known group validity showed that all subscales were able to discriminate between mild and moderate FD severity as classified by the FOS MISS (Main Severity Score Index). The FPHQ pain, heat and exertion subscale was responsive to change in symptoms between responders and non-responders as defined by change in EQ-5D index scores between Visits 1 and 2. CONCLUSIONS: The FPHQ is a valid and reliable tool for assessing patient-reported symptoms of FD. The questionnaire could be a useful tool for clinicians to understand the progression of disease and monitor treatment effects. FPHQ will be further validated and refined as the FOS database is continuously adding more patients.

PND46 DEVELOPMENT AND VALIDATION OF THE MULTIPLE SCLEROSIS RATING SCALE-REVISED (MSRS-R) Wicks F, Vaughan TE, Massaggi MP, Moldko, Cambridge, MA, USA
OBJECTIVES: PatientsLikeMe is an online health-data sharing community and research platform for patients with chronic and life-changing health conditions. In developing the PatientsLikeMe online platform for patients with Multiple Sclerosis (MS), we required a patient-reported, multi-dimensional assessment of functional status that was easy to complete. Existing measures of functional status were inadequate, clinician-reported, focused on walking, and burdensome to complete. To develop a longitudinal record accessible to patients using the site, we developed the Multiple Sclerosis Rating Scale (MSRS). METHODS: We adapted a clinician-rated measure, the Guy’s Neurological Disability Scale, to a self-report scale and deployed it to an online community. As part of our validation process, we reviewed online forum discussions between patients, conducted in-person patient cognitive debriefing, and made minor improvements to form a revised scale (MSRS-R). RESULTS: The MSRS-R self-report survey is a combination of self-reporting MS (RMMS) on the PatientsLikeMe platform. The survey included the MSRS-R as well as a range of comparator MS measures: PRIMUS, MSIS-29, PDSS, NARCOMS Performance Scales, and MSWS-12. RESULTS: In total, 816 RMS patients participated. The MSRS-R total high-intensity score: highest alpha = 0.86 and 1-week retest reliability (r = 0.91). The MSRS-R walking item was highly correlated with alternative walking measures (PDSS, r = 0.84; MSWS-12, r = 0.83; NARCOMS mobility question, r = 0.86). The MSRS-R correlated well with comparison instruments, and reliably differentiated between participants by PDSS disease stage, relapse severity, and time since diagnosis. Retrospective scoring of most recent relapse suggested a 3-point increase in MSRS-R might usefully identify relapses. CONCLUSIONS: The MSRS-R is a concise, multi-faceted measure of MS-related functional disability. It may be useful for describing the impact of MS and may further inquiry into the factors that relate to variation in outcomes among MS patients.

PND47 THE HUNTINGTON QUALITY OF LIFE INSTRUMENT (H-QOL-I): CROSS-CULTURAL VALIDATION IN GERMANY, POLAND AND USA Clay L1, Mirdad M2, Zielonka D2, Cohen J2, Toumi M3, Auquier P3
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OBJECTIVES: The Huntington Quality of Life Instrument (H-QOL-I) is a first self-reported specific Health-Related Quality of Life (HR-QoL) instrument developed to assess the QoL of patients suffering from Huntington’s disease. It was originally developed and validated in French and Italian. This study aimed to adapt and validated it in 11 languages. This study aims to validate the German, Polish and US versions of H-QOL-I cross-culturally. METHODS: The original questionnaire was based on 11 items and 3 dimensions. The instrument was translated forwards and backwards by native speakers. It was then reviewed and adjusted by local clinicians and tested for face validity. A survey was conducted with 134 US, 60 Polish and 41 German patients. Face validity was tested through item completion and overall understanding.