was designed to assess efficacy and safety of teriflunomide, a novel oral disease modifier, in RMS patients. The utility score, a measure of health-related quality of life, was calculated via the EQ-5D questionnaire assessed alongside the trial. 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 (RRMS) versus progressive relapsing (PRMS), region, number or relapses within the past 2 years, previous MS medication, gender, time (years) since first 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 RRMS and Eastern European countries versus North American countries. The major influencing factor, consistent with other analyses, was the EDSS score with the following disutility estimates: EDSS[1-2[=-0.021, p=0.52; EDSS[2-3[=-0.081, p=0.0128; EDSS[3-4[=-0.176, p<0.0001; EDSS[4-5[=-0.237, p<0.0001; EDSS[5-6[=-0.231, p<0.0001; EDSS[6-7[=-0.257, p<0.0001]. In addition, disutilities associated with PRMS versus RRMS and Eastern Europe region versus North American were respectively -0.073 (p=0.0028) and -0.038 (p=0.0361). **CONCLUSIONS:** In RMS patients, these results confirm the major impact of functional disability on patients' utility. These analyses also provided disutility estimates per EDSS score, disutilities for PRMS versus RRMS and for Eastern Europe versus North American region. The later probably reflecting cultural differences in health status perception.

PND37

AN EXAMINATION OF RESOURCE UTILIZATION AMONG PATIENTS WITH PARKINSON'S DISEASE TREATED WITH RASAGILINE OR SELEGILINE

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OBJECTIVES: To examine resource utilization associated with the use of Rasagiline or Selegiline, two commonly prescribed MAOB inhibitors for the treatment of Parkinson's Disease (PD). **METHODS:** Data for this retrospective study were obtained from the US i3 LabRx database over the time period from January 1, 2006 through December 30, 2010. Patients were included in the analysis if they were prescribed Rasagiline or Selegiline (with first such date identified as the index date), were diagnosed with PD (ICD-9 code 332.0), and had continuous insurance coverage from 6 months prior through 12 months post index date. Analyses are primarily descriptive in nature, with differences in categorical variables analyzed using chi-square statistics and differences in continuous variables analyzed using t-statistics. **RESULTS:** There were 1242 individuals included in the study - 926 initiated on Rasagiline and 316 initiated on Selegiline. Patients initiated on Rasagiline, compared to those intitiated on Selegiline, were significantly younger (63.2 years vs. 65.4 years; P=0.0020). Patients initiated on Rasagiline 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 intitated on Rasagiline were significantly more likely to visit a neurologist (93.63% vs. 89.24%; P=0.0105). Compared with Selegiline use, initiation on Rasagiline 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 who initiated therapy with Selegiline, compared to Rasagiline were more 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.

PND38

INTERNATIONAL COMPARISON OF HUNTINGTON DISEASE (HD) BURDEN

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OBJECTIVES: This study aimed to identify the socioeconomic burden of HD in five European countries **METHODS:** The survey was conducted in Germany, Italy, France, Poland and the USA. The following patient data were collected: clinical symptoms (motor, behavioral and psychiatric), functional/independence score, QoL (H-QoLI, SF-36, EQ-5D), resource utilization, GP and specialist visits, other healthcare professional visits, hospitalization, nursing home, social services, allowance, medical device and daily out of pocket expenses. The following data were collected from caregivers: time spent and working days lost caring for the patient, out of pocket expenditure, caregiver quality of life. **RESULTS:** To date, 175, 124, 44, 60 and 134 patients were included in respectively France, Italy, Germany, Poland and US. The populations were reasonably homogeneous regarding sociodemographic characteristics and severity such as age (48-56) and disease duration (6-10 years except for the Poland: 4 years). The average number of monthly visits to GP was 0.76-1.32, to neurologist 0.49-1.12, to physiotherapist 0.09-5.59. The percentage of patients admitted to hospital during the last 6 months was between 1% (USA) and 19% (France). The mean (± SD) health utility (EQ-5D) ranged from 0.25 (0.46) in France to 0.47 (0.37) in Germany. Caregivers spent between 6 (USA) and 22 hours/day (Italy, Poland) caring for patients and their monthly expenses amounted to €295 (Poland) to $2391 (USA). Caregivers also had reduced QoL. **CONCLUSIONS:** The initial results indicated significant differences in access to health care and resource use. France has the largest health care resource consumer by far. Countries that use little health care resources compensate by a significantly larger caregiver involvement. More data will be presented.

Neurological Disorders – Patient-Reported Outcomes & Preference-Based Studies

PND39

HEREDITARY ANGIOEDEMA HEALTH STATE UTILITY VALUATION STUDY FROM THE PERSPECTIVE OF A REPRESENTATIVE SAMPLE OF THE AUSTRALIAN GENERAL PUBLIC

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OBJECTIVES: The impact of hereditary angioedema (HAE) on patients' health related quality of life (QoL) extends beyond the acute attack period. This study was to value the disutility of living with HAE outside of the acute attack period, according to different emergency treatments available to the patient. **METHODS:** The study used a vignette/health state scenario based approach and standard gamble methodology. The health states described three different circumstances faced by patients with HAE in terms of availability of emergency medications should they suffer a swelling attack: Scenario A: HAE without any effective emergency medication; Scenario B: HAE with effective emergency medication available in hospital; Scenario C: HAE with effective emergency medication available for self-administration. The health state descriptions were based evidence from on relevant clinical trials, burden of disease and QoL studies and HAE treatment guidelines, supported by clinical expert opinion. The standard gamble survey was web based/administered online. Respondents were recruited from an existing consumer research panel. **RESULTS:** A total of 201 respondents completed the survey; 91% were prepared to gamble with death to achieve perfect health in at least one of the three health states. The mean utility weighting elicited for health state C was significantly higher than either weightings elicited for health state B (0.75 [95%CI. 0.71, 0.79] versus 0.64 [95% CI 0.60, 0.69]; p<0.001), or for health state A (0.75 [95%CI. 0.71, 0.79] versus 0.62 [95% CI 0.58, 0.67]; p<0.001). There was no statistical difference between the utilities elicited for health states B and A. **CONCLUSIONS:** The results demonstrate the recognition of and value placed on the QoL benefits provided by the availability of and immediate access to a self-administered emergency medication for HAE over that provided by treatment available only in the hospital accident and emergency treatment setting.

PND40

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

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OBJECTIVES: Health state 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 pain. This cross-sectional, observational study aimed to estimate utility values for different levels of migraine pain severity. **METHODS:** One hundred six participants from the UK (UK), diagnosed with migraine, completed the EQ-5D™ to evaluate their health status for mild, moderate, and severe levels of migraine pain severity for a recent migraine attack and for current health (without migraine) defined as health status within 7 days post-attack, with no residual migraine symptoms. T-tests were used to compare mean utility values between each level 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 found to be 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-5D 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), with 3 dimensions: motor function, psychology and socializing. It has been validated in several countries (France, Italy, Poland, Germany and United States). We compared several methods for mapping H-QoL-I onto EQ-5D and thus derive utility values from H-QoL-I. **METHODS:** This analysis was based on a sample of 315 HD patients who participated in an international survey on the burden of HD, and completed H-QoL-I and EQ-5D, with help from caregivers if necessary. EQ-5D index scores were calculated based on UK time trade-off tariff. The sample was divided into 70% derivation and 30% validation sets. We compared three methods to estimate patient's utility as a function of 11 H-QoL-I items: ordinary least-

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 estimated the mean EQ-5D score in the validation set (predicted score 0.32 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=0.0018), '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 fitting. Motor functions items contributed the model to utility predictions.

PND42

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 TERIFLUNONOMIDE PIVOTAL PHASE III TRIAL

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OBJECTIVES: In patients with RMS, assess the impact of relapse(s) leading to hospitalization 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 teriflunomide, a novel oral disease modifier, in RMS patients. Patients with no relapse, patients with relapse(s) not leading to hospitalisation and patients with at least one relapse leading to hospitalisation were analysed. The following patient 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 (CfB) in utility in patients with no relapse was +0.034, CfB in utility in patients with relapse(s) not leading to hospitalisation was -0.019(*p1<0.01) and CfB in utility in patients with relapse(s) leading to hospitalisation was -0.057(p1<0.001; p2:ns). Similar results were seen for PCS of +1.0, -1.0 (p1<0.01) and -3.1(p1<0.001; p2<0.05) respectively and for MCS were respectively +1.8, -1.0(p1<0.01) and -2.7 (p1<0.001; p2:ns). This same trend was observed with FIS total score, (CfB was respectively: -3.0, +1.4 (p1:ns) and +10.3 (p1<0.001; p2<0.01). 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.

PND43

VALIDATION OF THE SELF ASSESSMENT OF TREATMENT (SAT) QUESTIONNAIRE

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OBJECTIVES: The original SAT is a five-item questionnaire developed to assess treatment benefits associated with application of QUTENZA™, a novel high-dose capsaicin patch, in clinical trials among patients with neuropathic pain . The objective of this study was to evaluate the item performance and psychometric properties of the SAT. **METHODS:** The SAT, Numerical Pain Rating Scale (NPRS), SF-36, Brief Pain Inventory and Patient Global Impression of Change (PGCI) scores were measured in two 12 week Phase3 clinical trials. Descriptive statistics, exploratory and confirmatory factor analysis (EFA and CFE) were conducted to assess 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 analyzed. From descriptive statistics, EFA and CFA results, a one-factor model combining 4 of the 5 items emerged as the optimal solution with 66% 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 PGIC (0.85). SAT scores strongly discriminated patient change groups using the PGIC; 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 be further improved 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.

PND44

VALIDATION OF THE FABRY OUTCOME SURVEY (FOS) PAEDIATRIC HEALTH AND PAIN QUESTIONNAIRE

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OBJECTIVES: The original 40-question Fabry-specific Paediatric Health and Pain Questionnaire (FPHPQ) 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 FPHPQ. **METHODS:** FPHPQ 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 $\geq$ 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 $\geq$ 0.74). Construct validity was demonstrated by moderate correlation with the Brief Pain Inventory (BPI), KINDL, and EQ-5D. Known group validity showed that all subscales were able to discriminate between mild and moderate FD severity as classified by the FOS MSSI (Mainz Severity Score Index). The FPHPQ 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:** Preliminary analyses indicate that the measurement properties of FPHPQ are valid and reliable 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. FPHPQ will be further validated and refined as the FOS database is continuously adding more patients.

PND45

DEVELOPMENT AND VALIDATION OF THE MULTIPLE SCLEROSIS RATING SCALE-REVISED (MSRS-R)

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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). The MSRS-R was deployed as a cross-sectional survey to 4382 patients with relapsing-remitting MS (RRMS) on the PatientsLikeMe platform. The survey included the MSRS-R as well as a range of comparator MS measures: PRIMUS, MSIS-29, PDDS, NARCOMS Performance Scales, and MSWS-12. **RESULTS:** In total, 816 RRMS patients responded. The MSRS-R exhibited high internal consistency (Cronbach's alpha = 0.86) and 1-week retest reliability (r = 0.91). The MSRS-R walking item was highly correlated with alternative walking measures (PDDS, 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 PDDS 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 can support further inquiry into the factors that relate to variation in outcomes among MS patients.

PND46

THE HUNTINGTON QUALITY OF LIFE INSTRUMENT (H-QOL-I): CROSS-CULTURAL VALIDATION IN GERMANY, POLAND AND USA

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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 in Italian. The instrument is being validated 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 under-