HEALTH TECHNOLOGY ASSESSMENT FOR RARE DISEASES: A MARKOVIAN MODEL FOR FABRY DISEASE (FD) CONSIDERING ENZYME REPLACEMENT THERAPY (ERT)

**PSY62**

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**Objective:** Evaluate the current probabilistic model, the development of renal disease, the progression associated with the Disease (FD), comparing the probability of developing the different stages of renal disease in those with FD using Enzyme replacement therapy (ERT) with those not using ERT. METHODS: A model with predefined 3 possible stages was used for patients with FD and renal dysfunction. Progression to a different stage or having a stable disease were possible. Less severe stages could only migrate to more severe stages. Three year time cycles were used. The probabilities ERT x without ERT were compared. RESULTS: Probabilities for the 2 arms: 1. The probability of having proteinuria only and being stable in 3 y was 36% in those treated versus 48% in those untreated. 2. Also for those with proteinuria only, with ERT the probability of progressing to renal impairment in 3 y was reduced, to 24% compared to 32% in those untreated; and 3. For those receiving ERT already with renal impairment, but not in dialysis, the probability of postponing the dialysis in 3 y was not modified. CONCLUSIONS: This model showed a relevant improvement with ERT for those that were treated at initial stages of renal disease (the subgroup with proteinuria only). For this group the probability of remaining in a less severe stage of the disease in 3 y time, postponing renal impairment was significant for those using ERT. This was only a preliminary model, considering that the maintenance of less severe stages of renal disease postpones the need for dialysis in this sub-group, aspects such as quality of life will be added in order to do a more complete evaluation. This model was built considering clinical outcomes, something new on rare diseases. The discussion on how to evaluate and on the value of ‘classical modeling’ in rare diseases are still an on going issue with a lot to be elucidated.

WORK AND DAILY ACTIVITY IMPAIRMENT IN PATIENTS WITH DIGITAL ULCERS (DUS)—RESULTS FROM THE DUO REGISTRY

**PSY63**

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**Objective:** To assess outcomes in patients with DU disease associated with Scleroderma. The DUO Registry is an international, multi-centre, observational study to assess outcomes in patients with DU disease associated with Scleroderma. The results of a medical record review from eight hospitals in Spain will be presented. METHODS: A retrospective chart review of patients diagnosed with DU disease was undertaken at 8 hospitals in Spain, collecting demographic and clinical data, drug treatment (up to fifth line) and its duration. Thromboprotein-receptor agonists were not available at the time of the review. RESULTS: Data on 60 patients (53% (n = 38) with baseline platelet counts <30 x 10^9/L and 10% (n = 6) ≤50 x 10^9/L with bleeding episodes) were available. Patients were 54.4 ± 20.7 (SD) years of age, 61% female, and 6.0 ± 7.2 (SD) years since diagnosis. The most common first-line treatment was corticosteroids (n = 39, 65%), followed by intravenous immunoglobulins (IVIG; n = 19, 32%). The mean duration of first-line treatment with corticosteroids was 28.2 ± 39.0 (SD) days. The most common sequence of treatment for patients initially treated with corticosteroids was administration of four additional courses with corticosteroids (18/39, 46%), followed by alternation between IVIG and corticosteroids until the fifth-line of treatment (7/39, 18%). Of the 19 patients with initial IVIG, 5 (26%) received corticosteroids in the 4 remaining treatments, 5 (26%) continued with IVIG, and 4 (21%) alternated corticosteroids and IVIG. In total, 41 patients (68%) received 22 treatments with corticosteroids and 10 (17%) received treatments other than corticosteroids and IVIG. 3 patients received azathioprine, 3 rituximab, 2 platelet transfections, 1 anti-D immunoglobulin, 1 danazol and 1 methotrexate. CONCLUSIONS: Patterns of treatment of DU in Spain usually followed recently introduced recommendations from international consensus guidelines. However, in most patients, corticosteroid treatment was given repeatedly, which exceeds current recommendations, i.e., rapidly tapering corticosteroid dose and stopping after 4 weeks. Future research is needed with a larger sample size, to explore the place of splenectomy in treatment sequencing, and better understand the role of combination therapy.

SYSTEMIC DISORDERS/CONDITIONS – Conceptual Papers & Research on Methods

WEB VERSUS FACE-TO-FACE (FTF) ADMINISTRATION OF A HEALTH UTILITY SURVEY IN THE GENERAL PUBLIC: RESULTS FROM A TIME-TRADED (TTT) SURVEY ON IDIOPATHIC THROMBOCYTOPENIC PURPURA (ITP)

**PSY66**

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**Objective:** Web-based administration of health utility interviews offers the potential to recruit larger, more representative samples with reduced time and costs compared to FTF administration. This analysis compared health utilities elicited through web vs. FTF administration. METHODS: Six distinct ITP health states were included in a TTO and visual analogue scale (VAS)-based health utility valuation, which was administered FTF (n = 63) and via web (n = 319) to members of the UK general public. The Wilcoxon rank-sum test was used to compare utilities between methods. The interaction between administration method and respondent characteristics was assessed by regression analyses on each pair of health utilities. An additional analysis of exclusion criteria was conducted from least strict to most strict. RESULTS: Demographic characteristics in the TTF and web survey were generally comparable to the UK general population 2001 census data. The mean time to complete the TTO survey was 10.2 minutes in the FTF and 9.9 minutes in the web survey. Valid TTO response rates were higher in the FTF sample (85% to 96%) compared to the web sample (58% to 80%) across health states. Higher proportions of web respondents reported that the TTO exercise was ‘very’ or ‘somewhat unclear’ (17 % vs. none) that all or most decisions were difficult to make (41% vs. 30%) compared to the FTF sample. Utilities were statistically significantly lower in the web vs. the FTF survey (p < 0.05). TTO scores were sensitive to exclusion criteria (use or non-use of exclusion criteria) with TTF vs. web respondents. VAS ratings were similar across the two administration methods and less sensitive to exclusion criteria selection. CONCLUSIONS: Our study highlighted trade-offs between the advantages and challenges of web administration. More research is warranted to further improve data quality in web-based utility surveys.

PREDICTING EQ-5D UTILITIES FROM NEUROPATHIC PAIN SCORES: COMPARING INDIRECT MAPPING OF PREDICTED ITEM RESPONSES WITH DIRECT MAPPING OF SCORES

**PSY67**

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**Objective:** To predict EQ-5D utilities from neuropathic pain scores using indirect mapping of pain scores to EQ-5D item responses subsequently converted into utilities by direct mapping of pain scores into utilities. METHODS: Mappings were based on baseline data from three longitudinal surveys of adults (n = 2,719) who had ≥3 months of painful diabetic peripheral neuropathy (pDPN) or post-herpetic neuralgia (PHN), were receiving pain medications, and completed EQ-5D and pain questionnaires. In indirect mapping, ordered logit regression was used to predict and simulate EQ-5D responses using the following predictors: age, gender, and pain scores ranging from 0 (“no pain”) to 10 (“pain as bad as you can imagine”). Utilities were computed based on predicted responses using a U.S. algorithm. In direct mapping, OLS regression was used to directly predict utilities using the same predictors. Cross-validations were conducted separately in pDPN and PHN respondents. Comparisons were made between actual and estimated values on mean utilities, mean squared/absolute errors (MSAE/MAE). RESULTS: Both estimated utilities were consistently higher than actual values along the increment/decrement of the pain scores. Direct mapping explained 29% of the variance and had an estimated mean utility close to the observed data [0.594 (MSE = 0.31; MAE = 0.148)]. Indirect mapping resulted in lower mean utility [0.388 (MSE = 0.054; MAE = 0.184)] but its distribution was more consistent with the actual values.