were surveyed. SAS Version 9.1 (SAS Institute Inc, Cary, NC, USA) was used for data analysis. RESULTS: Patients with inhibitors showed higher rate (78%) and frequency (2.33 times per year) of hospitalization than those without inhibitors (42%, 1.13 times respectively). Also the rate of complications was higher (81%) compared with 59% for those without inhibitors. However, patients without inhibitors and those with inhibitors did not show statistically significant results in hospital visits, drug administration status, treatment-related tests (excluding blood tests), and adjuvant treatment. CONCLUSIONS: Patients with inhibitors are exposed to higher risk of bleeding and complications, which results in a more expensive medical utilization. In this regard, more active public subsidy program needs to be developed to prevent the generation of inhibitors.

MEASUREMENT OF HEALTH UTILITY VALUES IN THE UK FOR HEALTH STATES RELATED TO IMMUNE (IDIOPATHIC) THROMBOCYTOPENIC PURPURA (ITP)

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OBJECTIVES: The objective was to measure health states (HS) associated with Immune (Idiopathic) Thrombocytopenic Purpura (ITP), as perceived by members of the UK general public. METHODS: Six distinct ITP HSs were defined based on platelet level, bleeding symptoms, and key adverse events (bleeding complications). The data on bleeding and ITP-specific quality of life data were key sources for developing HS descriptions. After a pilot survey, sample size calculation was based on combinations of detectable differences in utility scores between HSs, power, and standard deviation (SD). A total of 593 respondents, who were randomly selected from a managed panel that includes 300,000 individuals in the UK, completed the web-based Time Trade-Off survey Sampling and tracking quotes on respondent demographic characteristics were performed to ensure representativeness. The Wilcoxon signed rank test was used to compare each pair of HS. RESULTS: Sample characteristics (mean age: 47.9 years, SD: 16.9; 54% female) were comparable to the UK general population. ITP HS were valued as significantly worse than perfect health. Experiencing episodes of bleeding was a more important driver than low platelet levels in valuing a HS to be worse. Substantial disparities were associated with surviving an acquired haemorrhage. Mean utility scores for the ITP HSs: HS1: Platelets ≤50x10^9/L, no outpatient bleed: 0.863 (SD:0.15) HS2: Platelets ≤50x10^9/L, outpatient bleed: 0.734 (SD:0.19) HS3: Platelets ≤50x10^9/L, no outpatient bleed: 0.841 (SD:0.19) HS4: Platelets ≤50x10^9/L, outpatient bleed: 0.732 (SD:0.19) HS5: Intracranial haemorrhage (24 months): 0.48 (SD:0.46) HS6: Steroid treatment adverse events: 0.758 (SD:0.20) CONCLUSIONS: This survey provided evidence that members of the UK general population associate a substantial loss of value living in ITP HS. These findings suggest an important role for new ITP treatments and utility scores gained from this survey can be used in future cost-effectiveness studies.

BLEEDING DISORDERS, MENORRHAGIA AND IRON DEFICIENCY: AN EXAMINATION OF QUALITY OF LIFE

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OBJECTIVES: Von Willebrand disease (VWD) is a bleeding disorder affecting about 1% of the general population. This study explored relationships between bleeding disorders, menorrhagia, iron deficiency and HRQL. It was hypothesized that females with bleeding disorders would have low HRQL associated with menstruation and iron deficiency. METHODS: All males and females with VWD, and females with other bleeding disorders, in the Canadian national registry, who were older than 12 years and literate in English or French, were eligible for survey in this cross-sectional study. A total of 1334 patients were recruited and completed a battery of questionnaires, including RMDQ, EQ-5D and SF-36v2 at the baseline, 1, 3 and 12-month follow-ups. The responses of SF-36v2 were converted into SF-6D using the published algorithm. Data were divided into two groups: one to estimate the mapping functions through regression methods and the other to validate the mapping functions. Both overall dataset and sub-datasets of each follow-up were examined separately RESULTS: Ordinary least square (OLS) regression with summary score of RMDQ as independent variable explained 27, 39, 46 and 53% of variance of observed EQ-5D scores for each follow-up, respectively. Based on the overall dataset, OLS regression explained 40 and 44 % of variance with the summary score and 18 items of RMDQ as independent variables, respectively. However, the mean absolute difference (MAD) of all above models was greater than 0.13. Meanwhile, models for mapping RMDQ into EQ-5D yielded an MAD of 0.02 to 0.04 of variance amongst all examined models and datasets. CONCLUSIONS: Based on the current findings, the score of RMDQ for back pain cannot be mapped into the SF-6D utility score. The possible cause could be the lack of over-lapping domains between two measures. The mapping of RMDQ into EQ-5D could be feasible but applied at the group level only. The correlation between RMDQ and EQ-5D increased when patients’ pain condition improved. Consequently, the choice of input dataset, overall or subset, has a great impact on estimating functions. The final selected dataset for mapping needs to be justified.

LINGUISTIC VALIDATION OF HAEMOPHILIA-SPECIFIC PATIENT-RATED OUTCOMES (HAEMO-QOL, HAEM-A-QOL, HEMO-SAT) IN UP TO 32 LANGUAGES

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Haemophilia is a congenital bleeding disorder with a prevalence of 1:10,000 inhabitants. Treatment of patients with haemophilia requires a substantial amount of economic and human resources, why patient-rated outcomes (PROs) such as Quality of Life (QoL) and treatment satisfaction (TS) are considered increasingly more important in this field. For the adequate assessment of PROs cross-cultural and disease-specific instruments are necessary. OBJECTIVES: Linguistic validation of 3 haemophilia-specific PROs for the assessment of TS (Hemo-Sat) and QoL, both in children (Haemo-Qol: 3 age group versions for children 4–7, 8–12, 13–16 years and their parents) and in adults (Haem-A-Qol) in up to 32 languages. METHODS: For languages where no translation existed, the process was conducted by a specialist in each target country using the following standardized methodology: (1) two forward translations by professional translators, native speakers of the target language and fluent in English; (2) comparison and reconciliation of the translations by the translators in the target country; (3) backward translation by a native English speaker; (4) comparison of source and backward version; (5) review by the developer for a selection of languages; creation of the different forms of the instrument; (6) review by a clinician. RESULTS: The Haem-A-Qol was linguistically validated into 32 languages, Haem-A-Qol (n = 29) and Hemo-Sat (n = 19). Besides the challenge of ensuring conceptual equivalence with the original and cultural appropriateness, the translation process revealed 2 additional difficulties. When translating an expression, appropriate terms had to be found for each age group while maintaining consistency across all versions of the same language. CONCLUSIONS: The language versions of Haemo-Qol, Haem-A-Qol and Hemo-Sat were established according to a rigorous translation methodology aiming to ensure conceptual equivalence across different language groups facilitating international comparison and pooling of data. The linguistic validation process is necessary before using PROs in international trials.

DEVELOPMENT OF A QUESTIONNAIRE TO EVALUATE TREATMENT SATISFACTION OF PATIENTS WITH SEVERE CROHN’S DISEASE: QUALITATIVE STEPS

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OBJECTIVES: Severe Crohn’s disease management includes anti-Tumour Necrosis Factor (anti-TNF) drugs. These drugs differ from the treatments used in early stages of the disease in terms of efficacy, safety and convenience. This can modify the impact of the disease and its treatment on patients’ lives. The objective of the study was to develop a questionnaire assessing patients’ satisfaction with anti-TNF treatments in Crohn’s disease. METHODS: A literature review, 3 clinician interviews and 3 interviews with nurses were carried out as a preparation step. Semi-directive patient...