and SCC. Average utilities for each health state were predicted from the regression coefficients, also estimated using individual health state dummy variables in a sensitivity analysis. RESULTS: Utilities for health states without BP or SCC were 0.76 for mild, 0.69 for moderate, 0.64 for marked, and 0.60 for severe. The utilities for states with bone symptoms were lower: 0.67 for mild+BP, 0.68 for moderate+BP, 0.66 for severe+BP, 0.63 for moderate+SCC, 0.52 for severe+SCC. Sensitivity analysis utilities were similar but less precise and sometimes less clinically plausible. CONCLUSIONS: Compared with the norm for the EQ-5D utility index in the UK (0.8), our survey results reflect the substantial burden of GD1, especially with skeletal complications. These utilities can be used in future economic evaluations of GD1 therapies to calculate quality-adjusted life years.

**PSY1**

**SOCIAL UTILITY VALUES FOR PEMPHIGUS VULGARIS AND FOLICACUS: A COMPOSITE TIME TRADE-OFF STUDY**

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OBJECTIVES: In the past few years, a number of biological drugs to treat pemphigus have become available. To estimate the health state utility weights for pemphigus vulgaris (PV) and folicacuse (FP) health states in a general population sample. METHODS: Three health state profiles were developed: an uncontrolled PV, an uncontrolled FP and a treated pemphigus state. Health state vignettes combined a description of living with pemphigus with summaries of the impact of the different severity levels of the disease on the clothing issues, and effects on work and social life. Utilities were obtained from a convenience sample of 106 adults using visual analogue scale (VAS, 0-10 scale) and time trade-off (TTO). Lead-time TTO was applied for health states considered worse than dead. Utilities in this study were ranging between -1 (lower anchor of worse than dead) and 1 (perfect health). RESULTS: Mean VAS scores for PV, FP and treated pemphigus were 6.15, 3.75 and 4.65, respectively. Significant differences were found between the three health states (P<0.001). CONCLUSIONS: This study provides the first utility values in pemphigus. Disutility associated with uncontrolled pemphigus was found worse than in uncontrolled psoriasis or atopic dermatitis. Successful treatment of pemphigus software. The design utilised 72 choices, (0.22-0.39). Utilities elicited in this study may be used in cost-effectiveness analysis of new pemphigus treatments.

**PSY2**

"FIRST AND FOREMOST BATTLE THE VIRUS" - PATIENT PREFERENCES IN ANTIVIRAL THERAPY FOR HEPATITIS C

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OBJECTIVES: Treatment options for patients with hepatitis C have made tremendous progress in the last 5 years. Innovative antiviral-therapies promise a huge reduction in the incidence of the disease and side-effects. Therefore, this is accompanied by an increasing focus on the patient's benefit. It is unclear, however, what properties generate the highest benefit for patients. METHODS: A systematic literature search was conducted to identify patients' preferences regarding antiviral therapies. Within N=24 semi-structured interviews, the HealthRef model was generated. Eight patient relevant characteristics were identified and described by three or six levels. For the discrete choice experiment, an experimental design of 32 and 39 profiles was generated using orthogonal main-effects and two-level interactions. RESULTS: The survey included 65 participants and the final model was estimated using choice-based conjoint analysis. Most relevant for patients were the side-effects and the duration of treatment. CONCLUSIONS: The results will be used to optimize the treatment of chronic hepatitis C and may be relevant for other chronic diseases.

**PSY3**

**TEN YEARS OF THE INTERNATIONAL POMPE SURVEY: PATIENT REPORTED OUTCOMES AS A RELIABLE TOOL FOR STUDYING TREATED AND UNTREATED CHILDREN AND ADULTS WITH NON-CLASSIC POMPE DISEASE**

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OBJECTIVES: Pompe disease is a rare, progressive lysosomal storage disorder for which currently no cure is available. The international Pompe survey (IPS) became available in 2006. Four years earlier, the IPS Erasmus MC survey, an international longitudinal prospective survey, was established to collect information on the natural course of the disease and its burden on patients. METHODS: The survey is a collaboration between Erasmus MC University Medical Center and the International Pompe Association (IPA) and comprises an annual questionnaire that was specifically designed to assess the symptoms and challenges related to Pompe disease. Utilities were estimated using individual health state dummy variables in a sensitivity analysis. RESULTS: Utilities for health states without BP or SCC were 0.76 for mild, 0.69 for moderate, 0.64 for marked, and 0.60 for severe. The utilities for states with bone symptoms were lower: 0.67 for mild+BP, 0.68 for moderate+BP, 0.66 for severe+BP, 0.63 for moderate+SCC, 0.52 for severe+SCC. Sensitivity analysis utilities were similar but less precise and sometimes less clinically plausible. CONCLUSIONS: Compared with the norm for the EQ-5D utility index in the UK (0.8), our survey results reflect the substantial burden of GD1, especially with skeletal complications. These utilities can be used in future economic evaluations of GD1 therapies to calculate quality-adjusted life years.

**PSY4**

THE IMPACT OF ORTHOPAEDIC SURGERY ON THE QUALITY OF LIFE OF HAEMOPHILIA PATIENTS WITH OR WITHOUT INHIBITORS: A SYSTEMATIC REVIEW OF THE LITERATURE

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OBJECTIVES: To conduct a systematic review of the literature and assess the impact of orthopaedic surgery on the health-related quality of life (HRQoL) of haemophilia patients with or without inhibitors. METHODS: Search of MEDLINE, EMBASE, EconLit, Cochrane library and the Center for Reviews and Dissemination databases from 2000 onwards. A broad search including both Medical Subject Headings (MeSH) and free text words related to haemophilia and orthopaedics was conducted. We included only the publications reporting pre- and post-surgery HRQoL data and all orthopedic interventions were considered. Congress abstracts from the American Society for Hematology and Plenary abstracts of the American Society for Blood and Blood Products were reviewed. Following the screening process, 8 full-text articles reporting pre- and post-surgery HRQoL data for patients who received ankle arthrodesis, total knee replacement, ankle replacement or hip arthroplasty were included. Seven studies used patient-reported measures and only one study estimated the EQ-5D-5L utility index as a gold standard procedure of indirect matching techniques of the seven studies, four used the visual analogue scale (VAS), three concluded that post-operative pain was significantly lower and only one study reported that although pain decreased post-surgery, moderate pain remained among patients who underwent multiple joint procedures and received ankle arthrodesis. Of the four studies that reported SF-36/SF-12 scores, significant improvements in physical health scores were observed in three and higher mental health scores were reported in one study. Disease-specific measures, Western Ontario and McMaster University (WOMAC) and Oxford Knee Score, were assessed by two studies and improvements in pain and physical functioning were observed on both scales. Evidence from SF-12 summary scores suggests that the improvements in physical functioning are similar for patients with and without inhibitors. CONCLUSIONS: Orthopaedic surgery improves the HRQoL of haemophilia patients with severe arthropathy.

**PSY5**

HEALTH STATES WORSE THAN DEAD IN AN ELDERLY POPULATION – PREVALENCE AND THE SIGNIFICANCE OF CHRONIC PAIN

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OBJECTIVES: When applying accepted health economic methods, it is not uncommon to find valuations of patients’ health states that are worse than dead. Hypothetical valuations (the UK value set) of states according to the EQ-5D resulted in 12.7 of 243 possible states being valued as worse than dead. The prevalence of health states valued worse than dead risks giving rise to counter-intuitive consequences when incorporated in health economic analyses. The purpose of this study was to therefor investigate the presence of such states in an exposed population, namely people 65 years and older. METHODS: Based on a Swedish epidemiological population survey study directed to 10 000 persons 65 years and older the occurrence of states worse than dead were investigated and related to prevalence and intensity of chronic pain. RESULTS: Health states worse than dead were found 1.8 % of the 6618 responders to the questionnaire. Among persons with severe chronic pain the prevalence was 16.7 % and among persons with moderate and no or mild chronic pain the prevalence of states worse than dead were 2.7 % and 0.8 % respectively. Prevalence of these states clearly increased with age and female gender. Of women 85 years and older with severe chronic pain 38.7 % were in states worse than dead. CONCLUSIONS: Health states worse than dead are not rare in an elderly population and covariate with chronic pain, age and female gender. The prevalence of states worse than dead are highly dependant on the value set applied, and will have consequences for assessments of treatments for elderly with chronic pain. Based on the results of this study it should be investigated whether and to what extent health states worse than dead exist when using experience based valuation methods, which is the preferred approach in some settings/countries.

**PSY6**

CLUSTERING HEALTH STATUS AMONG ADULTS WITH HEMOPHILIA USING SF-12 HEALTH SURVEY

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OBJECTIVES: Aims of the study were to: 1) conduct a study to investigate whether and to what extent health states worse than dead exist; and 2) to gain knowledge of the distribution of health status among hemophilia patients. One hundred and one patients between the ages of 18 and 72 were recruited and administered the SF-12 health survey. Internal consistency reliability and average scores for each health dimension were calculated. RESULTS: Of the 60 patients who completed the SF-12, 51.7% were classified as having a health status worse than dead. CONCLUSIONS: Health status worse than dead is a common health state among adults with hemophilia. Future research should explore the implications of these findings.
OBJECTIVES: To cluster health status among adults with hemophilia by applying the hidden Markov model (HMM) to account for longitudinal changes in quality of life scores and to derive utility weights for each cluster. METHODS: Data were obtained from the Hemophilia Utilization Group Studies (HUGS), a prospective, multi-center observational study conducted from 2005 to 2013. Demographic and clinical characteristics were collected. The hemophilia trial database was used to assess the quality of life (SF-12). A 3-month interview and 3-month follow-up visits for 2 years were collected. This analysis included data for 211 adults with hemophilia A or B with at least two observations. The HMM was applied to the SF-12 scores within HQUOL. Descriptive results showed that there were large mean differences in SF-36 domain scores for those with a diagnosis of pain vs. those without. The largest differences were observed for the bodily pain (M=40.91 vs. 51.88, p<0.001, d=1.06), general health (M=42.89 vs. 50.68, p<0.001, d=0.80), and role emotional (M=43.93 vs. 51.04, p<0.001, d=7.5) scales. With regard to pain severity in the past week, the strongest correlations were observed for the bodily pain (r=0.63, p<0.001) and role physical (r=0.46, p<0.001) scales. After controlling covariates, these associations still held for both the bodily pain (r=0.59, p<0.001) and role physical scales (r=0.45, p=0.03). The nature of the association between severity of pain (linear, quadratic) was also explored for each SF-6 domain score. CONCLUSIONS: Results suggest that the association between pain severity and HRQoL is nuanced and that pain affects different domains of HRQoL to varying degrees.

PSY90 ASSESSING THE IMPACT OF GROWTH HORMONE DEFICIENCY (GHD) IN CHILDREN: CONCEPT ELICITATION RESULTS SUPPORTING THE DEVELOPMENT OF THE TREATMENT-RELATED IMPACT-RELATED MEASURE FOR CHILDHOOD GHD (TRIM-CGHD)

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OBJECTIVES: Children with GHD can experience physical symptoms and disease-related impacts on functioning and well-being. However, no GHD-specific measures exist to assess these impacts. The purpose of this qualitative study was to collect data to support the conceptual validity of a new measure, with a patient-reported outcome (PRO) version for older children and an observer-reported outcome (OBO) version. METHODS: Focus groups and interviews were conducted with 39 children (aged 8–12) with GHD, 31 parents of children with GHD (aged 4–12) and eight clinical experts in three countries (Germany, UK, US). Interviews were analyzed and coded using adapted grounded theory to determine overarching themes and concepts. Based on the analysis, a conceptual model of the impact of GHD was developed and items for both versions generated and then cognitively debriefed.

RESULTS: Qualitative analysis found the saturation of concept-level reach was reached with four domains of impact: Symptoms, Physical, Social, and Emotional. Sub-concepts included appetite (48%), strength (42%) and energy level (38%) for Symptoms; limitations in physical performance (58%) and reaching (44%) for Social; worries for younger (50%) and treatment worries for older children (55%) and self-confidence (41%) for Emotional. Emotional impacts were often related to others’ perception or treatment of the child as younger. Children and parents reported consistent symptoms/impacts, although severity of impacts sometimes varied. Impacts were moderated by age and experience with GHD growth and age at treatment initiation. All versions were cognitively debriefed in a new sample (N=26: 13 children, 13 parents) and, based on findings, it was determined that the PRO version was appropriate for children aged 9–12. A 32-item TRIM-CGHD (PRO and OBO versions) was finalized.

CONCLUSIONS: The conceptual validity of both versions of the TRIM-CGHD is supported by these qualitative findings and the measure is now ready for psychometric validation.

PSY91 IMPACT OF WEIGHT LOSS ON PATIENT-REPORTED OUTCOMES IN THE SCALE OBESITY AND PREDIABETES TREATMENT OF LINagliotide 3.0 mg as ADJUNCT TO A DIET AND EXERCISE (D&E) PROGRAMME

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OBJECTIVES: To explore the impact of weight loss on patient-reported outcomes in individuals without type 2 diabetes, but with obesity (BMI ≥ 30 kg/m2) or overweight (BMI 27.0–29.9 kg/m2) with ≥ 3 comorbidities. Randomized 2:1 to once-daily linagliotide 3.0 mg (n=2487) or placebo (n=1244) as adjunct to D&E. METHODS: Impact of Weight on Quality of Life–Lite (IWQOL-Lite) and Short-Form 36 v2 (SF-36) questionnaire was administered at baseline and week 12 (2320 and 2153 individuals). Data were reported as estimated change at end-of-trial, derived using ANCOVA with LOCF; increased scores signify improvement. This post hoc analysis was performed to explore the relationship of weight loss (linagliotide 3.0 mg vs. placebo) acrossdomains in quality of life. RESULTS: Patients were randomized to linagliotide 3.0 mg, placebo respectively, as follows: A = weight gain, B = weight loss 0–4.9%, C = weight loss 5–9.9%, D = weight loss 10–14.9%, E = weight loss ≥ 15%. RESULTS: For individuals in the trial overall, greater proportions treated with linagliotide 3.0 mg versus placebo achieved weight loss categories C (30.1%, ≥ 16.5%), D (18.7%, ≥ 14.5%) and E (14.5%). For those completing the IWQOL-Lite (n=1890 linagliotide 3.0 mg, n=886 placebo) and SF-36 (n=1689, n=796 respectively), similar profiles were observed, with linagliotide 3.0 mg achieving higher categorical weight loss than placebo. Changes in IWQOL-Lite total score were lowest in the weight