OBJECTIVES: To cluster health status among adults with hemophilia by applying the hidden Markov model (HMM) to account for floor effects and for inter-individual changes in health status 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 included the presence of inhibitor, comorbidities and health-related quality of life (SF-36). Twenty interviews 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 modeled to account for floor effects and to fit the observed sequences, which consisted of SF-12 physical component scores (PCS) and mental component scores (MCS). Utility weights were derived using BRAHMS’s algorithm by mapping SF-12 scores to a SF-6D scale. RESULTS: Mean age was 34.6 ± 13.2 years and 95% had severe hemophilia. Data in four unique clusters provided the best fit with the observed sequences. Mean PCS were [54.2±2.5, 57.1±2.4], [50.4±6.5, 57.6±3.6], [32.4±7.7, 54.7±4.1] and MCS were [35.3±7.1, 36.8±6.8] from cluster 1 to cluster 4, respectively. Utility weights for each cluster were as follows: (A) 0.91±0.05, (B) 0.75±0.11, (C) 0.65±0.11 and (D) 0.55±0.08. At baseline, 49 (23%) adults were classified in cluster 1, 63 (30%) in cluster 2, 67 (32%) in cluster 3 and 32 (15%) in cluster 4. Being in a worse health cluster was significantly associated with unemployment, low household income, having severe hemophilia and being in cluster 1. CONCLUSIONS: Despite variability in health status, adults with severe hemophilia can be described by four mutually exclusive and clinically relevant clusters, which provide patients’ value of health outcomes for future economic analyses.

PSY7

OPIODS SWITCH IN CHRONIC PAIN: IMPACT ASSESSMENT USING ELECTRONIC RECORDS

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OBJECTIVES: Patients with chronic pain frequently require strong opioids for pain relief. While most patients achieve adequate analgesia, a significant minority either suffer intolerable side effects and/or inadequate pain relief leading to opioid withdrawal. The lack of a National Pain Care Registry does not permit a successful investigation in this topic. METHODS: Study population included all outpatient patients with chronic pain voluntarily participating in a cloud based medical surveillance ESUSAI. Scores (MCS). Utility weights were derived using Brazier’s algorithm by mapping SF-12 scores to a SF-6D scale. RESULTS: Mean age was 34.6 ± 13.2 years and 95% had severe hemophilia. Data in four unique clusters provided the best fit with the observed sequences. Mean PCS were [54.2±2.5, 57.1±2.4], [50.4±6.5, 57.6±3.6], [32.4±7.7, 54.7±4.1] and MCS were [35.3±7.1, 36.8±6.8] from cluster 1 to cluster 4, respectively. Utility weights for each cluster were as follows: (A) 0.91±0.05, (B) 0.75±0.11, (C) 0.65±0.11 and (D) 0.55±0.08. At baseline, 49 (23%) adults were classified in cluster 1, 63 (30%) in cluster 2, 67 (32%) in cluster 3 and 32 (15%) in cluster 4. Being in a worse health cluster was significantly associated with unemployment, low household income, having severe hemophilia and being in cluster 1. CONCLUSIONS: Despite variability in health status, adults with severe hemophilia can be described by four mutually exclusive and clinically relevant clusters, which provide patients’ value of health outcomes for future economic analyses.

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PSY8

VALIDATION OF A NEW HEMOPHILIA-SPECIFIC BURDEN SCALE FOR CAREGIVERS OF CHILDREN WITH HEMOPHILIA IN THE US – THE HEMOPHILIA ASSOCIATED CAREGIVER BURDEN SCALE (HEMOCABTM)

von Mackensen S1, Wasienski T2, Urso J1, Boggi L1

OBJECTIVES: Hemophilia is a hereditary bleeding disorder characterized by spontaneous and traumatic bleeding requiring regular or episodic infusion of factor VIII or IX concentrates. Although there are many instruments to assess the impact of this disease area, none have been validated specifically in the hemophilia population. We aimed to (i) develop a caregiver-reported outcome (PRO) version for older children and an observer-reported outcome version (ORO) for caregivers of children with hemophilia aged 0–12 years, (ii) have a panel of experts validate the content, (iii) conduct a psychometric analysis of the measure, and (iv) examine construct validity. METHODS: Focus groups and interviews with caregivers of children with hemophilia (CWH) 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). The conceptual validity of the measure is now ready for psychometric validation.

PSY9

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

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OBJECTIVES: GHD can experience physical symptoms and disease-related impacts on function and well-being. However, non-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 (ORO) version for caregivers. METHODS: Focus groups and interviews with caregivers of children with GHD (aged 4–12) and eight clinical experts in three countries (Germany, UK, US) were analyzed and coded using adapted ground 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 coherently defined. RESULTS: Qualitative analysis found the saturation of concept reached with four focus groups/panel (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 Physical function and emotional stress (60%), worries (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 factors such as linear growth status, body weight and age at treatment initiation. All versions were conceptually decribed 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 ORO 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 TRIAL OF LIRAGLUTIDE 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 one comorbidity. Patients were randomized to diet 2:1 to once-daily liraglutide 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 were administered at baseline and 3 months (80% of participants). Data were analyzed using ANCOVA with LOCF, increased scores signify improvement. This post hoc analysis was conducted using the primary endpoint, change in BMI (≥ 5%) and change in % weight loss (≥ 5%) in the liraglutide group. RESULTS: For individuals in the trial overall, greater proportions treated with liraglutide 3.0 mg achieved ≥ 5% weight loss than placebo. Changes in IWQOL-Lite total score were lowest in the weight

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