ISPOR Europe 2023

12 - 15 November

Poster Tour Guide Packet

Poster Session:	**Poster Session 1**
Tour Name:	**Patient-Centered**
Tour Date/Time:	Monday, 13 November 2023 | 11:30 - 12:15
Tour Location:	Area A, Poster and Exhibit Hall, Hall C

Acceptance Code:	PT1
Board Number:	1A
Abstract Title:	A Comparison of the UK and Australian Interim Value Sets for the Weight-Specific Adolescent Instrument for Economic Evaluation (WAItE)
Presenting Author:	Yemi Oluboyede

Abstract Body:

OBJECTIVES: The Weight-Specific Adolescent Instrument for Economic Evaluation (WAItE) is a weight-specific Health-Related Quality of Life (HRQoL) measure, containing seven dimensions with five severity levels. Two valuation studies (in the UK and Australia) have recently been conducted. This study aimed to compare the preferences from these valuation studies.

METHODS: Discrete Choice Experiments (DCEs) were conducted with adults in the UK (n=1,005) and Australia (n=1,005). DCE data were analysed using a mixed logit model. The interim UK value set was anchored onto the Quality Adjusted Life Year (QALY) scale using an external Time Trade-Off (TTO) study and the visual analogue scale (VAS). The interim Australian value set was anchored onto the QALY scale using the VAS method and the 'Comparisons with Death' (CWD) approach. Relative Attribute Importance (RAI) scores were calculated for both samples.

RESULTS: Inconsistencies were observed for certain dimensions in both the UK and Australian samples, and therefore some levels were combined to ensure monotonicity within dimensions. RAI scores showed the same dimension was valued as the least important in both samples, however, the most important dimensions differed. In the UK sample, the different anchoring methods generated similar value sets. Values for the 'PITS state' (the worst health state possible from the WAItE classification system) were 0.230 and 0.289 using the TTO and VAS anchoring methods respectively. Conversely, the interim value sets generated using each anchoring method in the Australian sample were quite different, as the values for the PITS state were quite different. The values for the PITS state were 0.429 and -0.030 when using the VAS and CWD anchoring methods respectively.

CONCLUSIONS: The UK and Australian interim value sets for the WAItE display several similarities, yet the values for the PITS state are not comparable. The choice of anchoring approach influences the results in both samples.

Tour Guide's Questions for Starting Q&A (Each poster will have ~5 minutes for Q&A with attendees/Tour Guide)

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Acceptance Code:	PT2
Board Number:	2A
Abstract Title:	Comparing UK Value Sets and Mapping Functions for EQ-5D
Presenting Author:	Johan Maervoet

Abstract Body:

OBJECTIVES: The EQ-5D is preferred by NICE to measure health-related quality of life. Versions with three (EQ-5D-3L) and five (EQ-5D-5L) response levels exist. Due to concerns about its methodology, quality and reliability, NICE does not recommend using the EQ-5D-5L value set for England published in 2018. To derive utility values from EQ-5D-5L responses, 5L data should rather be mapped onto the 3L value set. NICE previously recommended using the Van Hout crosswalk, but its 2022 Manual now states that the EEPRU mapping function developed by the Decision Support Unit (DSU) should be used. Our aim was to compare these different value sets and mapping methods.

METHODS: For each of the 3125 possible health states in the 5L system, utility values were obtained using the 2018 5L value set for England, the Van Hout crosswalk, and the DSU mapping function. In addition, utilities for the 243 possible EQ-5D-3L health states were calculated using the 3L value set for the UK, allowing comparison between corresponding 3L and 5L states. Density histograms and plots comparing utility values in comparable states were generated to visualize differences.

RESULTS: Utility values obtained with the 5L value set were generally higher than 3L value set/crosswalk estimates. Proportions of health states worse than death (utilities below zero) were 5.1% with the 5L value set, around 22% for the DSU mapping function (age and sex-dependent), 26.7% for the Van Hout crosswalk, and 34.6% for the 3L value set. Whilst the Van Hout crosswalk produces identical values for the 243 3L health states as the 3L value set, the DSU mapping function does not. Its utility values are slightly lower in the best health states, and generally higher in moderate and worst health states.

CONCLUSIONS: Utility values obtained with different UK value sets and mapping functions vary, potentially leading to differences in health-economic outcomes.

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Acceptance Code:	PT3
Board Number:	3A
Abstract Title:	Concept Elicitation Interviews to Refine a Conceptual Model of the Patient Experience of Amyotrophic Lateral Sclerosis (ALS)
Presenting Author:	Natalia Hakimi-Hawken

Abstract Body:

OBJECTIVES: Amyotrophic lateral sclerosis (ALS) is a rare, incurable neurodegenerative disease characterized by motor neuron loss resulting in weakness, disability, and eventually death. This study aimed to conduct qualitative interviews with people living with ALS exploring the signs, symptoms and impacts of ALS and refine a conceptual model (CM) for ALS.

METHODS: This study was a cross-sectional, non-interventional, qualitative study comprising of concept elicitation interviews with adult participants with a clinical diagnosis of ALS. Interviews were conducted by trained interviewers using a semi-structured interview guide. Interview transcripts were analyzed by directed content methods using ATLAS.ti software by trained researchers. Data collection and analysis was completed when data saturation was achieved.

RESULTS: Fifteen adult participants were interviewed. Twenty-two signs/symptoms were reported by participants, mostly spontaneously. Most frequently reported signs/symptoms of ALS were physical weakness (n=12/15, 80.0%), changes to speech/difficulty speaking/talking (n=11, 73.3%), respiratory/breathing issues (n=7/15, 46.7%), fatigue/tiredness (n=7/15, 46.7%), decrease in fine and gross motor control (n=7/15, 46.7%). The most frequently bothersome symptoms reported by participants included changes to their speech and talking, muscle spasms, twitches and cramps, breathing difficulties, and fatigue/exhaustion/lack of energy. Participants reported that ALS impacted many aspects of their physical functioning including difficulty walking, difficulty climbing stairs, difficulty eating. Participants experienced increased falls and needed to use mobility aids, due to reduced/loss of mobility. In addition to difficulty walking, most frequently reported bothersome impacts included emotional and mood impacts and loss of independence. A conceptual model, drafted after a review of existing literature/online blogs/forums was updated following patient interviews.

CONCLUSIONS: ALS is a debilitating and rapidly progressing disease with high unmet need and devastating impacts on all aspects of patients' lives with severe impacts on physical and emotional wellbeing. The conceptual model emerging from this study can be used to support the choice of existing disease-specific instruments in clinical studies.

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Acceptance Code:	PT4
Board Number:	4A
Abstract Title:	Patient-Reported Outcomes (PROs) in German AMNOG-Assessments: Impact of Increasing the Response Threshold to 15 %
Presenting Author:	Susan Fischer-Huchzermeyer

Abstract Body:

OBJECTIVES: Defining the Minimal Clinically Important Difference (MCID) for the evaluation of PROs is challenging. In March 2022, the Federal Joint Committee (G-BA) adopted a new response threshold of ≥ 15% of the scale range of the questionnaire for binary analyses of PROs. The aim of our study was to evaluate how the new response threshold affects the assessment of the added medical benefit of PROs in Germany.

METHODS: The G-BA website was searched for benefit assessments published in the transition period 01/2021-03/2023. The search terms were limited to EQ-5D, SF36, FACT as these questionnaires represent the most common PROs affected by the adoption. Only assessments including data on both analyses, the previously accepted MCID and the newly introduced threshold of ≥ 15%, and that were methodologically accepted by the G-BA were considered.

RESULTS: Overall, 23 of 129 screened assessments met the inclusion criteria. In 16 assessments, no significant PRO results could be demonstrated, regardless of the response threshold applied. For the remaining seven assessments, a significant treatment benefit was achieved with the previously accepted MID < 15%. However, in five of these seven assessments significant treatment benefit was also achieved with the new threshold of ≥ 15%.

CONCLUSIONS: Overall, only in seven assessments PROs achieved a significant treatment benefit. This demonstrates how challenging it is to achieve an added medical benefit through PROs. This might be explained by the fact that the majority of assessments (16/23) covered oncology drugs for which maintaining health status or quality of life is already considered a treatment success. The new response threshold of ≥ 15% represents a higher hurdle to prove an added medical benefit based on PROs. Consequently, minor but yet patient relevant benefits might not be valued appropriately.

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Acceptance Code:	PT5
Board Number:	5A
Abstract Title:	The Role of EUnetHTA 21 in Promoting Patient Engagement
Presenting Author:	Rachael Doran

Abstract Body:

OBJECTIVES: We aimed to evaluate patient engagement in the evolving European health technology assessment (HTA) process through exploring the current level of engagement and its impact, assessing the approaches taken by EUnetHTA to promote engagement, and identifying challenges and future perspectives.

METHODS: A targeted review of patient engagement in the HTA process was conducted via analysis of EUnetHTA deliverables, guidance documents, methodologies, and updates from EUnetHTA and European Commission websites.

RESULTS: Patient engagement in EUnetHTA initiatives is limited, from the perspective of stakeholders. However, EUnetHTA reported that patient input in early dialogue had valuable impact on engagement recommendations. In previous joint actions, EUnetHTA conducted 7 joint scientific consultations (JSCs), with 6 involving patients at the European level, and 2 joint clinical assessments (JCAs) for medical devices. The first published JCA mentioned that patients were consulted early in the scoping process. When establishing their current work plan, EUnetHTA and the European Medicines Agency prioritised development of methodologies for patient engagement in HTA. Deliverable D7.2/3 provides guidance for engaging patient representatives in HTA organisations. The European Regulation on HTA (HTAR) established a stakeholder network with 44 member organisations, including patient associations, and 2 observers. The EU4Patients project offers support by updating training content, designing an e-learning course, developing interactive training sessions for JCA and JSC, and implementing sustainability measures. Challenges remain, including lack of capacity and resources, expertise and training, alignment of organisations, conflict management, shared valuation of patient input, and pharmaceutical industry concerns regarding incorporation of stakeholder feedback.

CONCLUSIONS: Current patient engagement in EUnetHTA initiatives remains limited, despite efforts from EUnetHTA. HTAR presents a unique opportunity to enhance engagement. To strengthen this framework and ensure efficient HTA, potential future measures could include promoting and allocating resources for patient training projects and establishing communication channels between national patient groups across Europe.

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Acceptance Code:	PT6
Board Number:	6A
Abstract Title:	What Do Patients Value? Development of a Preference-Based Health Utility Score for Chronic Obstructive Pulmonary Disease (COPD)
Presenting Author:	Byron Jones

Abstract Body:

OBJECTIVES: There is increasing interest in the integration of quantitative evidence from patient preference studies into HTA decision-making with a question being how this can best be achieved. We generated a health utility score for patients with COPD and considered its use within HTAs.

METHODS: Based on prior qualitative research, six symptoms were identified as important to COPD patients: shortness of breath, exacerbations, chronic cough, mucus secretion, sleep disturbance and urinary incontinence. A Discrete Choice Experiment (DCE) based on these six symptoms was employed with 1050 COPD patients from 5 countries (US, UK, France, Australia, Japan). The random parameter logit regression technique was used to estimate utility scores for all COPD health states. The relationship between patients' COPD health utility score, self-perceived severity of their COPD and EQ-5D-3L utility score was assessed, with data stratified according to patient COPD disease severity and comorbidity subgroups.

RESULTS: The COPD health utility model had face validity, with utility scores negatively correlated with patients' self-perceived COPD severity. Correlation between the COPD health utility scores and EQ-5D-3L values was only moderate (Spearman correlation, 0.52). This was largely explained by patients with EQ-5D-3L scores <0 exhibiting a range of comorbidities beyond their COPD which impacted their EQ-5D-3L scores, whereas the COPD health utility score was impacted less by the comorbid conditions.

CONCLUSIONS: A disease-focused measure of utility offers benefit in clinical trials of new therapies and in generating utility data in support of HTA submissions. Our COPD utility measure, derived from the DCE, provides a patient-centered health utility score, which is more sensitive to the COPD health of the individual and less sensitive to comorbidities. The instrument should be considered alongside more generic instruments for use when valuing new COPD therapies and using utility data in submissions to licensing and reimbursement agencies.

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