### **Poster Tour Guide Packet**

Poster Session:	In-Person and Virtual Poster Session 2
Tour Name:	Methodology Research in HEOR
Tour Date/Time:	Monday, 7 November 2022, 2022, 17:45 - 18:30
Tour Area:	Area B, Hall X2, Level -2

Acceptance Code:	MSR30
Board Number:	1B
Abstract Title:	Linguistic and Psychometric Validation of the "Cognition" Bolt-on Version of the Japanese EQ-5D-5L (EQ-5D-5L+C) By Adding a Cognitive Dimension for the Elderly
Presenting Author:	Ataru Igarashi

#### Abstract Body:

OBJECTIVES: To develop a cognition bolt-on version of the Japanese 5-level EQ-5D (EQ-5D-5L+C) by referring to a previously published bolt-on version, we linguistically validated a cognitive dimension and psychometrically validated the EQ-5D-5L+C.

METHODS: Following linguistic validation of the cognition dimension performed according to a standard process, psychometric validation utilized data collected from residents of six nursing homes between Oct-2021 to Jan-2022 for its main study. The EQ-5D-5L modification and its use were granted by the EuroQol group. Anonymized data of background information, EQ-5D-5L+C and EQ-5D-5L (both proxy versions responded by the nursing home staff), and Mini-Mental State Examination (MMSE) at baseline and month 3 were drawn. Validity, reliability, and sensitivity were evaluated.

RESULTS: The Japanese EQ-5D-5L+C was finalized after forward and back translations of the cognition dimension and cognitive interviews where 5 professional/family caregivers answered and understood the cognition dimension along with the Japanese EQ-5D-5L without major difficulty. Data of 254 participants was analyzed for the psychometric validation. Mean (± standard deviation) age and Barthel Index scores at baseline were 87.14±7.29 years and 52.47±30.58, respectively, with 68.9% being women and donepezil being most frequently prescribed (7.5%).No ceiling or floor effects were found in the cognition dimension. For construct validity, the strongest correlation was found between the cognition dimension and MMSE scores (rs=-0.640). The test-retest reliability in the cognition dimension between two time points was good (k=0.644, 95% confidence interval [CI]: 0.541–0.746). Although the sensitivity to change was weak between changes in the cognition dimension and MMSE score (rs=-0.191 [95%CI: -0.325--0.058]), adding the cognition dimension to EQ-5D-5L benefited in capturing MMSE score changes.

CONCLUSIONS: The Japanese proxy version of EQ-5D-5L+C is a valid tool to capture health status including cognitive function in the elderly nursing home residents in Japan, with a careful consideration required to capture cognitive changes.

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:	MSR52
Board Number:	2B
Abstract Title:	Target Trial Emulation (TTE) for Real World Data Analyses to Support HTA Decisions
Presenting Author:	Alastair Bennett

#### Abstract Body:

OBJECTIVES: To introduce Target Trial Emulation (TTE) methods for the analysis of real-world data (RWD) to support HTA decision making and demonstrate their application to derive causal average treatment effect estimate for survival outcomes from RWD with risk of bias from immortal-time and time-varying confounding.

METHODS: We analyse longitudinal patient level data from the EU Myelodysplastic Syndrome (EUMDS) registry to quantify the causal average treatment effects of alternative protocols for using erythropoiesis-stimulating agents (ESA) as first line therapy in intermediate-1 to low-risk Myelodysplastic Syndrome patients. We apply causal inference methods for survival outcomes with time-varying exposures for static interventions using a TTE framework. We constructed (target trial) protocols to assess alternative treatment modalities and used a 'clone and censor' approach to account for the risk of immortal time bias. Inverse probability of censoring weights were calculated to address the induced selection bias and time-varying confounding. We compared naïve and weighted Kaplan Meier (counterfactual) curves for the alternative treatment strategies.

RESULTS: We found that ESA is only beneficial for the first year with survival difference of 1.1% (-4.2% – 6.4%). However, towards the end of follow up at 6 years we found a difference in survival of -3.1% (-16.4% - 10.2%). A sensitivity analysis based on an alternative study protocol for the use of ESA in this patient's population revealed a large benefit for ESA in terms of survival throughout the entire follow up period with a survival difference of 3% (-2.8% - 8.9%) at year 1 to 13.6% (-2.5% - 29.9%) at year 6.

CONCLUSIONS: The recently launched NICE RWE framework highlights the opportunities of using RWD to support HTA. Our case study shows how to use causal inference methods to emulate a target trial and produce relevant estimates of treatment effect which can inform a clinical and funding decisions.

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Acceptance Code:	MSR70
Board Number:	3B
Abstract Title:	A Head-to-Head Comparison of the EQ-5D-5L and 15D Descriptive Systems and Index Scores in a General Population Sample
Presenting Author:	Valentin Brodszky

#### Abstract Body:

OBJECTIVES: The EQ-5D-5L and 15D are generic preference-accompanied health status measures with similar dimensions. In this study, we aim to compare the measurement properties of the EQ-5D-5L and 15D descriptive systems and index scores in a large general population sample.

METHODS: In August 2021, an online cross-sectional survey was conducted in a nationally representative adult general population sample in Hungary (n=1887). The EQ-5D-5L and 15D descriptive systems and index scores were compared in terms of ceiling and floor effects, informativity (Shannon's evenness index), agreement, convergent validity and known-groups validity for 20 different chronic physical and mental health conditions. Danish value sets were used to compute index scores for both instruments.

RESULTS: Among the corresponding dimensions, both the ceiling and floor effects were smaller for the EQ-5D-5L in most dimension pairs (exceptions: EQ-5D-5L anxiety/depression vs. 15D distress for the floor effect, and EQ-5D-5L anxiety/depression vs. 15D depression for the ceiling effect). The 15D index scores showed smaller ceiling effect than the EQ-5D-5L (21% vs. 36%). The average informativity was better for the EQ-5D-5L dimensions (0.56 vs. 0.49). A strong correlation was found between the EQ-5D-5L and 15D index scores (r=0.671). In most cases, the EQ-5D-5L was able to better discriminate between healthy respondents and those with chronic diseases; however, the difference in relative efficiency was insignificant in 18/20 condition groups (exceptions: thyroid diseases and gastroesophageal reflux disease).

CONCLUSIONS: This is the first study to compare the measurement properties of the EQ-5D-5L and 15D in a general population sample. The EQ-5D-5L performs better than the 15D for most measurement properties. Our findings help to understand the differences between the EQ-5D-5L and 15D instruments and index scores and provide broad information for health economic evaluations and resource allocation decisions.

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Acceptance Code:	PCR4
Board Number:	4B
Abstract Title:	Disease-Specific Quality-of-Life Measures Mapped to the EQ-5D in a Chronic Disorder With Recurrent Attacks: Analysis of the Deliver Trial in Patients With Migraine
Presenting Author:	Linus Jönsson

#### Abstract Body:

OBJECTIVES: Recall period is important when selecting appropriate patient-reported outcomes (PROs) for generating health utilities. People living with migraine experience variation in their daily experience, which may not be well captured using generic quality-of-life measures such as the EQ-5D with its 1-day recall period. In this post hoc analysis of the DELIVER (NCT04418765) migraine-prevention trial, we compared utilities estimated from 3 PRO measures—EQ-5D-5L, Migraine-Specific Quality of Life Questionnaire (MSQ; 4-week recall), and 6-item Headache Impact Test (HIT-6; 4-week recall) to assess the extent to which these measures capture migraine impact. The relationship between utilities and monthly migraine day (MMD) reduction was also examined with the 3 PRO measures.

METHODS: MSQ and HIT-6 were mapped to EQ-5D-3L using previously published algorithms. Utilities were estimated for each study visit (up to 24 weeks) at which the HIT-6, MSQ, and/or EQ-5D-5L was completed. A mixed linear model comparing number of MMDs and utility score was estimated for each instrument. The base-case model estimated the relationship between utilities and MMDs independent of treatment group.

RESULTS: Analysis of MSQ and HIT-6 showed a similar relationship with changes in MMDs (average utility decrement per MMD [95% CI]: MSQ, 0.0189 [0.0180, 0.0198], p<0.001; HIT-6, 0.0188 [0.0181, 0.0195], p<0.001). Relative to MSQ and HIT-6, EQ-5D-5L was less responsive to MMD changes (0.0053 [0.0048, 0.0059], p<0.001). When pooling or stratifying patients (n=337) by migraine type, a linear correlation between migraine presence during the day of EQ-5D-5L completion, migraine severity, and scores obtained using EQ-5D-5L was observed: presence and severity impacted the utility estimate (values ranged from 0.62–0.92).

CONCLUSIONS: Measures with a short recall period may not fully capture migraine's impact on a patient's life and functioning. Thus, this work highlights the importance of investigating different approaches that may be used to generate health utility data.

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Acceptance Code:	PCR200
Board Number:	5B
Abstract Title:	Measuring the Burden of Alopecia Areata with the European Quality of Life-5 Dimensions (EQ-5D): Results from a Real-World Survey in 5 European Countries
Presenting Author:	Erin Johansson

#### Abstract Body:

OBJECTIVES: Alopecia areata (AA) is an autoimmune condition that has been associated with a negative impact on quality of life (QoL) and increased prevalence of anxiety and depression. This analysis aimed to describe the burden of AA according to physician-rated current severity using EQ-5D.

METHODS: In October 2021, an online survey began in the United Kingdom (UK), France, Germany, Italy and Spain. Dermatologists actively treating patients with AA were recruited. They completed questionnaires for 7 consecutive adult patients with mild, moderate, and severe AA. Current disease severity was rated based on physician clinical judgement. Data on patient demographics were collected. Patients were invited to complete a paper self-completion form (PSC): including EQ-5D-5L questionnaire and visual analogue scale (VAS) (range, 0 [worst] to 100 [best] imaginable state of health). An index value was derived from the EQ-5D-5L (range, 0 [worst health] to 1 [best health]). Health state indices were derived from country-specific value sets. Data from patients who completed a PSC are reported using an interim sample (March 7, 2022).

RESULTS: A total of 532 patients with AA were included (mild n=91 moderate n=267; severe n=174). Overall, 56.0% were males and mean age was 34.3 years (±standard deviation [SD] 10.72; range 18 to 72). Patients with mild, moderate, and severe AA reported a mean±SD EQ-5D value of 0.90±0.10, 0.85±0.14 and 0.78±0.17 (UK value set), respectively. Results from the EQ-5D anxiety/depression dimension showed that 67.0% of patients with severe AA reported feeling anxious and/or depressed on the day of their completion compared with 44.0% and 49.0% of patients with mild and moderate AA, respectively. Patients with mild, moderate, and severe AA reported a mean±SD VAS score of 82.2±13.38, 74.3±14.89 and 68.0±19.73, respectively.

CONCLUSIONS: Patients with severe AA reported lower QoL and higher anxiety and/or depression than patients with mild and moderate AA, as measured by EQ-5D.

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Acceptance Code:	PCR3
Board Number:	6B
Abstract Title:	Assessing Concept Saturation and Sample Representativeness When Using Social Media Data to Inform Concept Elicitation Studies
Presenting Author:	Bill Byrom

#### Abstract Body:

OBJECTIVES: Social media (SM) data, such as that posted on online health boards (OHBs), can provide insights to aid concept elicitation in the development of clinical outcome assessments, and other application areas. While SM data samples are often much larger than those achieved in cognitive interview studies, understanding concept saturation remains important. In addition, OHB users are often younger and include greater proportions of females compared to many patient populations, making it important to consider the representativeness of the sample when drawing conclusions.

METHODS: We use OHB posts to elicit understanding of meaningful aspects of physical activity in CHF patients. We used autocorrelation between concepts identified over time to measure the convergence of findings to quantify concept saturation. Further, we implemented weighting adjustments to adjust for the differences in age distributions between our cohort and the CHF population.

RESULTS: We used 383 posts from 271 individuals with CHF. Ages were determined for 209/271 (77%) of individuals, and were normally distributed (range: 20 – 96, mean: 54, SD: 15 years). Adjusting findings non-parametrically for the true population age distribution did not change the overall conclusions, but increased the importance of certain concepts including "self-care" and "going out", while lessening concerns around "productivity" and "sick leave". Saturation analysis showed asymptotic growth in identified concepts, but correlation between the overall frequency distribution of concepts from post to post began to stabilise after the first 30 posts, and continuing to code beyond the first 90 posts had negligible impact on the overall findings of the study.

CONCLUSIONS: SM data may contribute to concept elicitation knowledge important in outcome measure development. The methods we describe help to ensure that findings are representative of the patient population, and that concept saturation is established. These approaches contribute to enhancing the robustness of findings from social listening studies.

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