

### PMU93 THE VALUE OF PATIENT PREFERENCE STUDIES IN HEALTH TECHNOLOGY ASSESSMENTS IN EUROPE

Young KE, Guillaume X, Bonnellye G  
Kantar, Paris, France

**Objectives:** Due to the lack of guidance on how to optimize evidence demonstration and systematically incorporate quantitative patient preference studies in HTA, the study aimed to investigate how patient preference data may be supplied, its relevance in HTA, and how it has been valued in UK, France and Germany. **Methods:** Secondary desk research including HTA websites, peer reviewed articles, and grey literature was done. Three case studies were identified with the aim of identifying differences in the type and use of patient preference data, how preference data is developed, and for which purpose across indications. **Results:** The three case studies covered oncology, rare metabolic, and infectious disease indications. In all cases, patient preference studies were incorporated in phase 3 trials and qualitative methods were used by means of treatment preference or satisfaction questionnaires. The attributes assessed included efficacy, side effects, mode of administration, convenience, and overall satisfaction. All patient preference data supported the primary or secondary endpoints, and QoL data. In the HTA reports, patient preference data were not assessed to replace neither clinical nor economic evidence. In contrary, it provided complementary insight on the relevance of the clinical outcomes in the daily life of a patient who suffers from the disease. **Conclusions:** The relevance of patient preference data may rest on its purpose in how it supports the overall value proposition and the evidence base of the treatment that is relevant to the patient. Through providing complementary data that only patient insight can provide, it may help HTA committees in evaluating and deliberating the clinical and economic evidence in reimbursement decision making. Manufacturers should not delay the preference of patients until after launch. Planning and implementation should happen as early as Phase 2-3, with appropriate and robust methodologies for measuring and linking data to validated clinical and economic outcomes.



### PMU94 UTILITY MEASUREMENT BY TIME TRADE-OFF METHOD IN HUNGARY: A SYSTEMATIC LITERATURE REVIEW

Balázs PG,<sup>1</sup> Brodzsky V,<sup>2</sup> Rencz F<sup>3</sup>

<sup>1</sup>Corvinus University of Budapest, Doctoral School of Business and Management & Department of Health Economics, Budapest, Hungary, <sup>2</sup>Corvinus University of Budapest, Department of Health Economics, Budapest, PE, Hungary, <sup>3</sup>Hungarian Academy of Sciences, Premium Postdoctoral Research Programme & Corvinus University of Budapest, Department of Health Economics, Budapest, Hungary

**Objectives:** Time trade-off (TTO) is a widely used method to assess health utilities for economic evaluations of health technologies. We intended to systematically review and synthesize all available studies using TTO method in Hungary. **Methods:** In January 2020, we conducted a systematic literature search in three electronic databases (Medline, Web of Science and the Hungarian Periodicals Table of Contents Database). Our inclusion criteria were: (1) original publications, which (2) measured utilities using TTO, (3) on a Hungarian sample. **Results:** Nine original publications were included, in a total of seven chronic diseases (age-related macular degeneration, chronic migraine, Crohn's disease, pemphigus, primary dysmenorrhea, psoriasis and rheumatoid arthritis) reporting utilities for 23 different health states. Each study used a self-administered mode of administration (six paper-based surveys and three online surveys). The sample size ranged from 86 to 1808 respondents. Four studies used general population samples, four used patient groups and one study used both. Seven out of nine (77%) studies followed a conventional TTO with better than dead scenario and two (22%) used the composite TTO method, which had a worse than dead scenario too. The most frequently used timeframe was 10 years (44%). Mean utilities in the studies ranged from 0.34 for uncontrolled pemphigus vulgaris to 0.94 for mild primary dysmenorrhea. The proportion of non-traders varied between 0% and 29% across studies. **Conclusions:** An increasing number of TTO studies are available in chronic diseases based on preferences of the Hungarian population or patients. TTO utilities summarized in this review may be used to support local data-driven health technology assessments in Hungary. Methodological standardization and representative samples are suggested in future TTO studies.



### PMU95 A SYSTEMATIC LITERATURE REVIEW OF PREFERENCE STUDIES IN HAEMOPHILIA

Morgan G,<sup>1</sup> Martin A,<sup>1</sup> Mighiu C,<sup>1</sup> Sagar A,<sup>1</sup> O'hara J,<sup>1</sup> Sawyer EK,<sup>2</sup> Li N<sup>2</sup>

<sup>1</sup>HCD Economics, Daresbury, UK, <sup>2</sup>UniQure, Inc., Lexington, MA, USA

**Objectives:** Prophylactic infusions of the missing clotting factor (Factor VIII or Factor IX) is the current standard of care for people with haemophilia (PwH). Novel therapies for haemophilia include bispecific antibody, non-factor therapy, and gene therapy. The objective of this study was to examine treatment attributes included in previous preference studies to inform the design of a discrete choice experiment (DCE) to examine preferences of novel therapies for haemophilia. **Methods:** A



systematic literature review (SLR) of electronic databases, including MEDLINE<sup>®</sup>, psycINFO, and Cochrane Library, was conducted (04/2005-04/2020). Relevant conferences were also queried. Two reviewers independently double-screened studies according to pre-defined criteria. Data were then extracted, including study population, design, statistical approach, and treatment attributes described in the DCE design or health state description. **Results:** Of 2296 titles and abstracts screened, 15 relevant studies were identified for review. All studies focused on factor replacement therapy. Of 15 studies, 13 (86.7%) DCEs and two (13.3%) time trade-off studies were identified. Sample sizes ranged from 30 to 600. Preferences were derived from patients, caregiver, healthcare professionals, and general population in 12 (80.0%), 9 (60.0%), 6 (40.0%), and two (13.3%) studies, respectively. The most common attributes assessed included treatment frequency (14 [93.3%]), effect on bleeds (12 [80.0%]), and risks and side effects (9 [60.0%]). **Conclusions:** Existing preference studies in haemophilia suggest that treatment frequency, effectiveness, risks and side-effects are the key attributes for factor replacement therapy. Further research is needed to define and understand the preferences for relevant attributes associated with novel therapies for haemophilia, including gene therapy.

### PMU97 EXPLORING ANTIMICROBIAL RESISTANCE (AMR) FROM A SOCIETAL PERSPECTIVE: PREFERENCES AND WELFARE IMPACTS IN THE UNITED KINGDOM

Dorgali MV,<sup>1</sup> Longo A,<sup>2</sup> Vass C,<sup>3</sup> Shields G,<sup>3</sup> Harrison R,<sup>3</sup> Boeri M<sup>4</sup>  
<sup>1</sup>University of Florence, Florence, Italy, <sup>2</sup>Queen's University Belfast, Belfast, UK, <sup>3</sup>The University of Manchester, Manchester, LAN, UK, <sup>4</sup>RTI Health Solutions, Belfast, UK

**Objectives:** In the past century, antibiotics have led to a gain in life expectancy of approximately 20 years. However, over time, antimicrobial resistance (AMR) is accelerating, becoming a global public health concern with serious health and financial consequences: if nothing is done, estimates suggest AMR could cause 10 million deaths and cost \$100 trillion annually by 2050. Therefore, understanding societal preference for the use of antibiotics, as well as eliciting the willingness to pay (WTP) for future antimicrobial drug development, is crucial. The objective of this study was to investigate individuals' preferences for different strategies to contain AMR employing discrete choice experiments (DCE). **Methods:** A DCE was administered to a sample of the UK adult population. Respondents were asked to make 9 choices between 2 hypothetical "doctor and antibiotics" scenarios and 1 "no doctor – no antibiotics" option defined by five attributes: treatment, days needed to recover, risk of bacterial infection which needs antibiotics, risk of common side effects and risk of AMR by 2050. Data were analyzed using random-parameters logit models. **Results:** 2,436 respondents completed the survey. The risk of AMR by 2050 was the most important attribute being 3.8 (0.74÷0.195) times as important as days needed to recover; although, when presented with a higher risk of bacterial infection, respondents placed a lower importance on the risk of AMR by 2050. In a follow up contingent valuation question to estimate the monetary value of AMR policies, we also found an average WTP of ~£60 annually for 5 years per household. **Conclusions:** The risk of AMR is relevant and important. The aggregate WTP for containing AMR is about £5.7 billion, and with society's preferences for the attributes of future approaches to combat AMR, should help policy makers develop future AMR strategies.



### PMU98 A LITERATURE REVIEW OF THE IMPACT OF DISEASE OUTBREAKS AND NATURAL DISASTERS ON HEALTH-RELATED QUALITY OF LIFE

St Pierre D,<sup>1</sup> Lin X,<sup>2</sup> Kosinski M<sup>3</sup>

<sup>1</sup>Optum, Cranston, RI, USA, <sup>2</sup>Optum, Johnston, RI, USA, <sup>3</sup>GSK, Collegetown, PA, USA

**Objectives:** To identify patient-reported outcome measures (PROs) of health-related quality of life (HRQoL) used in studies of disease outbreaks and natural disasters; to examine the impacts of such crises on HRQoL; and to inform the study design of future studies of crises. **Methods:** A literature review was conducted following a pre-specified protocol. PubMed was searched for publications reporting the impacts of disease outbreaks and natural disasters on HRQoL, assessed by PROs, using Medical Subject Headings (MeSH) terms and keywords: disease outbreaks (MeSH) + infectious, natural disasters (MeSH), quality of life, HRQoL, and health status. Studies were included if they provided a method of comparison, such as pre- and post-crisis comparisons (within-subjects), or using an unexposed control group (between-subjects). After two rounds of screening, information was extracted on study characteristics, PRO(s) used, and impacts on HRQoL. **Results:** 226 abstracts were retrieved from the PubMed search; 15 met the criteria for inclusion. Five focused on disease outbreaks (SARS, MERS, H1N1, and COVID-19) and 10 on natural disasters (hurricanes, earthquakes, tsunamis, wildfires, and avalanches). Nine studies used an unexposed control group design and 7 studies used a pre- and post-crisis design. Multiple PROs were identified that captured HRQoL, including the EQ-5D and SF-36. Compared to control groups, people exposed to crisis reported lasting HRQoL impairment, particularly in mental health domains. In several of the longitudinal studies reviewed, pre-crisis measures of mental health predicted post-crisis impacts

