HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG)
E-Bulletin, December 2019

Enhanced quality and relevance of HTA through patient and citizen involvement

http://www.htai.org/interest-groups/patient-and-citizen-involvement.html

Welcome to our E-Bulletin and 2020!

From our Chair

As we wrap up another productive year, I'd like to thank all of you working in different capacities, different stakeholder groups and different jurisdictions to further the work of involving patients in HTA. Difference can divide people, but not in PCIG. Valuing being informed by different needs, preferences and experiences has led many of us to patient involvement in HTA, and it's the same value that provides a strong foundation for the multi-stakeholder work of this Interest Group.

I'd like to express my thanks to all who are working on PCIG projects. Through our projects PCIG fosters change by developing practical products, guidance and journal articles. Project delivery is central to PCIG and so I'm deeply appreciative of the extra hours and days project members give on top of already heavy workloads.

Additionally, I'd like to thank Rebecca Trowman for all the support she has given PCIG during her time with HTAi. I'm delighted that although Rebecca is leaving the HTAi Secretariat, she will continue to be involved in PCIG. And, finally, I'd like to thank Janney Wale who is putting together this e-bulletin as most of us in Australia are packing up and heading to the beach.

Wishing you all good health and happiness in 2020.

Ann Single, Chair – HTAi Patient and Citizen Involvement Interest Group
singlehaworth@gmail.com

If your membership is up for renewal, please visit this page to get started, and so you can still access this E-Bulletin.

HTAi Matters

HTAi 2020 Annual Meeting Beijing
Attaining, maintaining and sustaining healthcare systems in a changing World: the role of HTA HTAi2020.org

Early registration opens mid-January 2020. Acceptance notifications for abstracts due February 1, 2020.

Travel grants https://www.htai2020.org/travel-grants-2/
Successful/Unsuccessful Application Notification: February 3, 2020

Plenaries https://www.htai2020.org/the-role-of-hta/and plenaries
https://htai2020.org/speakers-2/
Plenary one: The Role of HTA in Achieving and Progressing Universal Health Coverage
Panelist Dr Ratna Devi is CEO and co-founder of DakshamA Health and Education in India, an organisation that is dedicated to working for access to health, patient education and advocacy. DakshamA aims to create a network of caregivers and patient groups, and work with them on knowledge sharing as well as providing essential feedback for managing long term and chronic diseases. She leads a cross disease Patient Alliance in India called Indian Alliance of Patient Groups As a strong patient safety advocate, she works with government as well as patient organisations to advocate for better quality and safety at home, educational institutions, and workplaces.
Ratna has more than 30 years of experience working to improve health outcomes in India and is present Chair of International Alliance of Patients' Organizations (IAPO).
Kalipso Chalkidou, Director of Global Health Policy for the Centre for Global Development, based in London, is to chair this plenary. Her work concentrates on helping governments build technical and institutional capacity for using evidence to inform health policy as they move towards Universal Healthcare Coverage. She is interested in how local institutions can drive scientific and legitimate healthcare resource allocation decisions.

Plenary Two: How to Adapt HTA to Address Technologies that are 'Disrupting' Health Systems
Professor Gillian Leng, Deputy Chief Executive, NICE, United Kingdom will chair the second plenary. Among her many roles Gillian chairs the national Shared Decision Making Collaborative and sits on several national boards. She is actively involved in research into quality improvement, is a trustee at the Royal Society of Medicine, and chair of the Guidelines International Network.
Durhane Wong-Rieger is a Panelist for the plenary. Durhane is President and CEO of the Canadian Organization for Rare Disorders, Chair of Rare Disease International, Vice-Chair of Asia Pacific Rare Disease International, and member of the Editorial Board of The Patient- Patient Centred Outcomes Research Institute. She is a member and a former Chair of the PCIG, as well as a former Chair of IAPO.

Plenary Three Incoming Tides and What it Means for HTA; the Rise of Real-World Evidence, 'Big Data', and Artificial Intelligence.
Dr Noor Hisham Abdullah is a Panelist for this plenary. He is a Senior Consultant Surgeon in Breast and Endocrine Surgery, Putrajaya Hospital and the current Director General of Health Malaysia. His special interest lies in enhancing universal healthcare coverage to marginalized communities and bringing personalized healthcare back to the community and home through digital technology and creative innovation.

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The HTAi Ethics Interest Group held a writing and planning workshop 18-20 December in Oslo, Norway
Nine Interest Group members met to continue work started at the Interest Group's 2018 Amsterdam meeting and to discuss the Interest Group's program of activities for the 2020 HTAi Annual Meeting in Beijing, and to begin planning for the 2021 HTAi meeting. The main outputs from the Oslo meeting are draft manuscripts on core competencies for ethics in HTA and ethical issues involved in disinvestment decisions. The workshop was made possible through Interest Group funding from HTAi.

What's Happening

New Australian website launched to support PBAC consumer comments
The Patient Voice Initiative has a new online resource to help patients and the wider community submit Consumer Comments to the Pharmaceutical Benefits Advisory Committee (PBAC). The intent is that comments are more likely to inform PBAC'S advice to the Minister on listing medicines.

Designed to complement Department of Health information, the new website, www.patientvoiceintiative.org, guides users through the way PBAC works and the specifics of what patients know that may address gaps and uncertainties in the evidence being considered by the Committee.
The Patient Voice Initiative built the site in consultation with the Australian Department of Health's Consumer Evidence and Engagement Unit (CEEU) with multi-pharma sponsorship after providing training workshops for patients and carers in each of the state capitals.
In 2020, the site will be further developed to include an expanded library of shareables for social media and guidance to contributing to the Medical Services Advisory Committee and developing robust patient-based evidence.

Submitted by Ann Single

FDA and Real World Evidence (RWE) - Use of RWE to support the approval of new indications for drugs already approved, or to help support or satisfy post-approval study requirements
In December 2018, FDA published a Framework for its real world evidence (RWE) program. FDA's evaluation will consider: whether the real world data (RWD) are fit for use; whether the trial or study design used to generate RWE can provide adequate scientific evidence to answer or help answer the regulatory question; and whether the study conduct meets FDA regulatory requirements.
Within FDA, a steering committee of Center leadership and subject matter experts has overseen policy development and provided resources and guidance to review divisions evaluating novel applications for use of RWE including exploring new methodologies to collect and curate RWD that address data quality and relevance challenges.
Externally FDA has been actively engaging stakeholders in various forums, for example, FDA has held two public meetings: the first on leveraging RWD in randomized clinical trials, and the second an annual review of considerations for developing RWE for regulatory use including initiatives to improve and assess data quality, use of mobile technologies and sensors, and evaluation of observational methods to draw causal inference about product effectiveness.
Random treatment assignment and other methods to control bias will likely remain critical to generation of reliable evidence of effectiveness. FDA understands that great interest exists in exploring whether non-interventional (observational) designs (designs where treatment is assigned during clinical practice, and analytic techniques are then used to assemble 'comparable' populations and control for potential confounders that may drive clinical treatment selection, can support a finding of product effectiveness.
FDA's approach will also consider flexibilities needed to allow for the consideration of variable RWD sources such as from EHR, claims data, and data captured using digital tools.

From DIA - FDA's Real World Evidence Program Moving Forward
https://globalforum.diaglobal.org/issue/december-2019/fdas-real-world-evidence-program-moving-forward/?utm_medium=email&utm_source=db&utm_content=PUB_GF_Dec_2019-12-21A&utm_campaign=globalforum&utm_type=aq&mkt_tok=eyJpIjoiTUdNMo56VXdZVGM1TkdRMSIsInQiOiJDVohkco0ZKYTVpMUIxZ09iT1paYkR6Z1BJRHlheDRvQitrVnFpbW9FY2IycHFFVmx3K2k2MlpKNnJhZzBFUlByZXJRRlwvSlRId1loZmM2Tm52czNKVDB3Y1FoZzNqUGp3clVEUm5WbmZ3czE0YzhwTWRzU21KUEZhYnRQc2tHbHMifQ%3D%3D

ICER Evaluation for curative treatments?

In the US, there has been some discussion over whether curative treatments, just like other new drugs, could undergo Institute for Clinical and Economic Review (ICER) evaluation. In other words, is it reasonable to price curative medicines based on the degree of expected long-term improvement in patient health? After reviewing Zolgensma, ICER concluded that despite the uproar over price, the drug is in fact cost-effective. Nevertheless the fear is that the market may wrongly translate these conclusions to mean that ALL gene therapies can be indiscriminately priced within a $1-$2 million range, which would be disastrous for the US health system that's expecting 20+ more of these therapies over the next five years.
ICER has evaluated another three FDA-approved gene and cell-based therapies. Kymriah (tisagenlecleucel; Novartis) and Yescarta (axicabtagene ciloleucel; Kite Pharma/Gilead), which were developed for relapsing or refractory B-cell lymphoma and have the price tag of $475,000 and $373,000, respectively. They were found to be fairly priced.
However, Luxturna (voretigene neparvovec; Spark Therapeutics), which is for the treatment of retinal disease and costs $850,000, was way above ICER's evaluation; its price needs to be reduced by at least 50% to meet cost-effectiveness thresholds. ICER's pricing recommendations have led the way to price falls before: Novartis initially considered a figure approaching $5 million for Zolgensma but settled on a price in accordance with ICER's recommendations.

ICER recognizes that its framework isn't the answer to valuing curative treatments and there is a growing need to learn how to effectively evaluate the prices of single or short-term therapies in a way that enables both patient access and manufacturing feasibility. Consequently, it has announced a new project where they are collaborating with methodology experts, international Health Technology Assessment (HTA) groups such as the UK's National Institute for Health and Care Excellence (NICE) and the Canadian Agency for Drugs and Technologies in Health (CADTH), and other stakeholders to consider if methods for evaluating curative medicines should differ from methods for evaluating ongoing maintenance medications.
Several academic and government non-profit organizations are also working to tackle a complexity that stems from various factors but primarily uncertainty about the efficacy and durability of curative treatments, and the side effects. Health systems are also structured around delivering maintenance medications for chronic conditions and are currently ill-equipped to handle the core challenges posed by single and short-term treatments.
https://eloqua.eyeforpharma.com/LP=26007?utm_campaign=EFP%2013DEC19%20Newsletter%20Everyone%20Else&utm_medium=email&utm_source=Eloqua&elqTrackId=57e15407dba24e4497d4de646352fd28&elq=5b7cd71cf7cb435da967a90ff3ea7679&elqaid=49832&elqat=1&elqCampaignId=30939

Publications

Addario B, Geissler J, Horn MK, et al. Including the patient voice in the development and implementation of patient-reported outcomes in cancer clinical trials. *Health Expect*. 2019;00:1–11. https ://doi.org/10.1111/hex.12997
The review article considers a holistic view of how the patient voice can be used in clinical trials and serves as a practical guide to help researchers optimise patient-reported outcomes in clinical trials.

Rocco Falchetto. The Patient Perspective: A Matter of Minutes
10th Meeting "International Academy of Health Preference Research" Basel, Switzerland, 13–14 July, 2019. Patient 2019;12(4):429–35 https://doi.org/10.1007/s40271-019-00399-2 This article describes how patient input can contribute to medical product research and development processes, regulatory reviews, health technology assessments and reimbursement decision-making.
At the European Medicines Agency (EMA) meeting in September 2014, regulatory approval for afamelanotide to treat adults with erythropoietic protoporphyria (EPP), an ultra-rare inborn disease that causes severe intolerance to light, was discussed. At the symposium, Professor Hillege mentioned that it was one of the few cases to his knowledge whereby a meeting with patients overturned a unanimous negative opinion. Five years after the EMA approval, patient access to afamelanotide remains limited.
Rocco is a porphyria advocate and biochemist, and has EPP. The manufacturer of afamelanotide submitted an application to EMA for marketing authorization in early 2012. The porphyria patient community was full of hope and eagerly looking forward to a rapid approval of the drug. Naturally, the EMA had little knowledge of the severity and life-limiting nature of EPP, a poorly understood ultra-rare disease burdened by the additional challenge of 'invisibility'. To overcome this understandable hurdle, the porphyria patient community actively engaged with the EMA, offering to educate the agency about EPP and the benefits experienced by patients receiving afamelanotide treatment. After numerous exchanges, in April 2014, an EPP patient and carer delegation finally had the opportunity to speak at an EMA ad hoc expert group meeting. In September 2014, two patients with EPP were invited to the plenary meeting of the EMA Committee for Medicinal Products for Human Use (CHMP) to discuss the benefits and risks of afamelanotide. On 24 October, 2014, the EMA recommended afamelanotide for marketing authorization under exceptional circumstances, recognizing the challenges in assessing the efficacy and the lack of therapeutic alternatives in EPP. The lack of scientific instruments, such as specific biomarkers, to objectively measure the extent of phototoxic reactions is the first challenge to assessing the clinical efficacy of therapeutic candidates in EPP. Deeply ingrained light avoidance is another. The ultra-rarity of EPP limits the number of patients that can be enrolled in clinical trials. A 5-year period was experienced by patients all over Europe to access the treatment since the EMA approval in 2014, hampered by how the clinical trial data were expressed. It is approved in Germany.
Patient preference knowledge and realworld evidence has been gathered during the 5 years since EMA approval contributed to another key milestone, on this 14-year-long journey: reviewers at the US Food and Drug Administration unanimously approved afamelanotide to treat patients with EPP on 8 October, 2019.

Emma J. Cockcroft, Nicky Britten, Linda Long, Kristin Liabo. How is knowledge shared in Public involvement? A qualitative study of involvement in a health technology assessment. Health Expectations. First published: 29 November 2019. https://doi.org/10.1111/hex.13001

Submitted by Nicky Britten

New issue of International Journal of Technology Assessment in Health Care:
Franz Pichler, Wija Oortwijn, Alric Ruether, Rebecca Trowman. Defining capacity building in the context of HTA: a proposal by the HTAi Scientific Development and Capacity Building Committee. IJTAHC Volume 35, Issue 5, 2019, pp. 362-366. DOI: https://doi.org/10.1017/S0266462319000631

Neill Booth. On value frameworks and opportunity costs in health technology assessment. IJTAHC Volume 35, Issue 5, 2019, pp. 367-372. DOI: https://doi.org/10.1017/S0266462319000643

Sharing two more manuscripts on patient preferences in dry eye disease
- first one is the consolidated data and methodology (self-explicated conjoint); and the second paper examines country differences and preferences of moderate and severe patients:
Asbell P, Messmer E, Chan C, et al. Defining the needs and preferences of patients with dry eye disease. BMJ Open Ophthalmology 2019;4:e000315. doi:10.1136/bmjophth-2019-000315
Methods and Analysis. Following a literature review and social media listening (step 0), qualitative phone call interviews were conducted with 12 patients (step 1). Patients' responses underwent content analysis and were coded, quantified and displayed as charts. Based on the emerging trends and attributes identified as relevant in steps 0 and 1, a quantitative online questionnaire was designed and conducted with 160 patients across four countries (step 2).
Results. The online questionnaire was rated as easy/very easy to understand by 60% of respondents, 62% rated the survey as easy/very easy to complete and 71% rated it as interesting/very interesting. Treatment satisfaction was the most important aspect for patients, and the three most relevant attributes were as follows (with the most important indexed to 100%): 'treatment effectiveness on symptoms of dry eyes' (100%), 'frequency of treatment use' (96%) and 'how the treatment works' (95%).
Conclusion. Our methodology was well received by patients, and the results will help inform future clinical trial development and discussions with health technology assessment bodies and regulators on unmet needs and product attributes that are of most value to patients with dry eye disease.

Messmer E, Chan C, Asbell P, et al. Comparing the needs and preferences of patients with moderate and severe dry eye symptoms across four countries. BMJ Open Ophthalmology 2019;4:e000360. doi:10.1136/bmjophth-2019-000360
A quantitative questionnaire was developed based on the self-explicated conjoint methodology and was administered to 160 patients with moderate or severe dry eye disease (DED) from Australia, Germany, UK and the USA.
Results Patients with moderate dry eye symptoms ranked 'treatment satisfaction' as the most important aspect, whereas 'symptom bother' was more relevant for those in the severe group. Both the moderate and severe groups classified treatment effectiveness as the most important treatment attribute. This result was consistent across the four countries, although US patients gave significantly higher scores than patients from other countries ($p<0.001$). Furthermore, patients from Australia ranked 'treatment experience' as significantly more important than the concern of side effects, whereas respondents from Germany exhibited the opposite trend ($p<0.05$ for both). The health burden of DED is reflected in the average European Quality of Life-5 Dimensions 5-level (EQ-5D) scores of 0.764 and 0.658 for patients with moderate and severe disease, respectively.
Conclusion Our results confirm that across the countries in the study, moderate and severe DED has a major impact on patients' quality of life and daily activities. By providing insight into the patient perspective of DED, our study helps identify outcomes that are important to patients and may guide future drug development and clinical decision-making.

Submitted by Nigel Cook

PREFER (Patient Preferences in Benefit and Risk Assessments during the Treatment Life Cycle), a public–private research initiative – publications
E de Bekker-Grob; J Juhaeri; U Kihlbom; B Levitan. Giving patients' preferences a voice in the medical product lifecycle: why, when and how?: The public-private PREFER project: Work package 2. ISPOR Value & Outcomes Spotlight, 2018, Vol 4, No 3, pp 19-21.
PREFER is a five-year project funded equally by the Innovative Medicines Initiative (IMI; Europe's largest public-private initiative aiming to speed the development of better and safer medicines for patients) and by industry as in-kind contribution. IMI is a partnership between the European Union's Horizon 2020 program and the European pharmaceutical industry represented by EFPIA (the European Federation of Pharmaceutical Industries and

Associations). This paper describes the structure of the project, the work package devoted to answering when and how patient preferences should be considered in the medical product life-cycle.

R Janssens; I Huys; E van Overbeeke, Eline et al. Opportunities and challenges for the inclusion of patient preferences in the medical product life cycle: a systematic review. BMC Medical Informatics and Decision Making, online 4 October 2019. This systematic review of peer-reviewed and grey literature aimed to understand the potential roles, reasons for using patient preferences (PP) and the expectations, concerns and requirements associated with PP in industry processes, regulatory benefit-risk assessment (BRA) and marketing authorization (MA), and HTA and reimbursement decision-making. Most identified documents were written from an academic perspective (61%) and focused on PP in BRA/MA and/or HTA/reimbursement (73%). PP was used to improve understanding of patients' valuations of treatment outcomes, patients' benefit-risk trade-offs and preference heterogeneity. Reasons for using PP relate to the unique insights and position of patients and the positive effect of including PP on the quality of the decision-making process. Concerns shared across decision-making contexts included methodological questions concerning the validity, reliability and cognitive burden of preference methods.

R Janssens; S Russo; E van Overbeeke et al. Patient Preferences in the Medical Product Life Cycle: What do Stakeholders Think? Semi-Structured Qualitative Interviews in Europe and the USA. Patient, 2019 vol 12, issue 5, pp 513-526. This study used semi-structured interviews to characterize stakeholders' attitudes, needs, and concerns with respect to patient preferences (PP) in decision making along the medical product lifecycle, from seven European countries and the USA. Interviews were conducted between April and August 2017. Interviewees reported being unfamiliar (43%), moderately familiar (42%), or very familiar (15%) with preference methods and studies.
Despite the interest all interviewed stakeholder groups reported in PP, the effective use of PP in decision making across the medical product lifecycle is currently hampered by a lack of standardization and consensus on how to both measure and use PP.

C Whichello; E van Overbeeke; R Janssens et al. Factors and Situations Affecting the Value of Patient Preference Studies: Semi-Structured Interviews in Europe and the US. Frontiers in Pharmacology, online 18 September 2019
This study conducted semi-structured interviews with six different stakeholder groups (physicians, academics, industry representatives, regulators, HTA/payer representatives, and a combined group of patients, caregivers, and patient representatives) from seven European countries (the United Kingdom, Sweden, Italy, Romania, Germany, France, and the Netherlands) and the United States to identify factors and situations that influence the value of patient preference studies (PPS) in decision-making along the medical product lifecycle (MPLC) according to different stakeholders. Fifteen factors affecting the value of PPS in the MPLC were identified. These are related to: study organization (expertise, financial resources, study duration, ethics and good practices, patient centeredness), study design (examining patient and/or other preferences, ensuring representativeness, matching method to research question, matching method to MPLC stage, validity and reliability, cognitive burden, patient education, attribute development), and study conduct (patients' ability/willingness to participate and preference heterogeneity). Three types of situations affecting the use of PPS results were identified (stakeholder acceptance, market situations, and clinical situations).

V Soekhai; C Whichello; B Levitan et al. Methods for exploring and eliciting patient preferences in the medical product lifecycle: a literature review. Drug Discovery Today, 2019, Volume 24, Issue 7, pp 1324-1331.
We developed a compendium and taxonomy of preference exploration (qualitative) and elicitation (quantitative) methods by conducting a systematic literature review to identify these methods. This review was followed by analyzing prior preference method reviews, to cross-validate our results, and consulting intercontinental experts, to confirm our outcomes. This resulted in the identification of 32 unique preference methods.

S Russo; C Jongerius; F Faccio et al. Understanding Patients' Preferences: A Systematic Review of Psychological Instruments Used in Patients' Preference and Decision Studies. Value in Health, 2019, Volume 22, Issue 4, pp 491-501, 2019. This study set out to assess which psychological instruments are currently used and which psychological constructs are known to have an impact on patients' preferences and health-related decisions - including the formation of preferences and preference heterogeneity. From 33 studies, we identified 33 psychological instruments and 18 constructs, and categorized the instruments into motivational factors, cognitive factors, individual differences, emotion and mood, and health beliefs. Our results indicate that measures of health literacy, numeracy, and locus of control have an impact on health-related preferences and decisions.

E van Overbeeke; C Whichello; R Janssens et al. Factors and situations influencing the value of patient preference studies along the medical product lifecycle: a literature review. Drug Discovery Today, 2018. Volume 24, Issue 1,

January 2019, pp 57-6. In general, experience in conducting and assessing patient preference studies is limited. Here, we performed a systematic literature search and review to identify factors and situations influencing the value of patient preference studies, as well as applications throughout the medical product lifecyle. We identified possible applications in discovery, clinical development, marketing authorization, HTA, and postmarketing phases.

E de Bekker-Grob; C Berlin; B Levitan et al. Giving Patients' Preferences a Voice in Medical Treatment Life Cycle: The PREFER Public–Private Project. Patient, 2019, Volume 10, Issue 3, pp 263–266
Treatments are developed for patients, and there is an emerging consensus that patients should be involved at crucial decision points in the treatment life cycle. In general, stakeholders (i.e., industry, regulatory authorities, health technology assessment [HTA] bodies, reimbursement agencies, clinicians, and patient organizations) all agree about the importance of incorporating patients' preferences, needs, and perspectives into decision making and the need to provide more avenues for patient engagement. Combining a multi-disciplinary approach with a consortium of various stakeholders is essential, allowing urgent and relevant questions to be answered and giving patients' preferences appropriate roles in the treatment life cycle.

E van Overbeeke, R Janssens, C Whichello C et al (2019). Design, Conduct, and Use of Patient Preference Studies in the Medical Product Life Cycle: A Multi-Method Study. Front. Pharmacol. 10:1395. doi: 10.3389/fphar.2019.01395
Stakeholder perspectives on how PPS should be designed and conducted: 1) study design should be informed by the research questions and patient population; 2) preferred treatment attributes and levels, as well as trade-offs among attributes and levels should be investigated; 3) the patient sample and method should match the MPLC phase; 4) different stakeholders should collaborate; and 5) results from PPS should be shared with relevant stakeholders.
Four applications seemed most promising for systematic integration of patient preferences: 1) benefit-risk assessment by industry and regulators at the marketing-authorization phase; 2) assessment of major contribution to patient care by European regulators; 3) cost-effectiveness analysis; and 4) multi criteria decision analysis in HTA.

Submitted by Nigel Cook

Whitty JA, de Bekker-Grob EW, Cook NS, Terris-Prestholt F, Drummond M, et al. Patient preferences in the medical product lifecycle. Patient. 2019. https ://doi.org/10.1007/s4027 1-019-00400 -y.
From a symposium on "Patient preferences in the medical product lifecycle" in Basel, Switzerland in July 2019, jointly sponsored by the International Academy of Health Preference Research (http://www.iahpr .org) and the research project "Patient Preferences in Benefit and Risk Assessments during the Treatment Life Cycle" (PREFER; http://www.imi-prefe r.eu).

1 Does Patient Preference Information in Reimbursement Decision -Making Lead to Higher Quality Decisions and Increased Public Acceptance of Decisions Regarding Allocation of Healthcare Resources?
Undertaking preference studies early in the development process is an opportunity to strengthen their impact on both the development process and reimbursement.

2 What Should Happen First to Achieve Successful Integration of Patient Preference Information into the Medical Product Lifecycle?
Regulators look to industry to generate good-quality preference studies to support their decision making. However, pharmaceutical and medical technology companies may be averse to spending research resources to undertake studies that are not seen to influence regulators' and/or HTA body decisions.

3. Which Preference Methods are Most Promising or Acceptable to be Used in Regulatory Assessments or Reimbursement Decision Making?
Currently, discrete choice experiments are perceived as the gold standard for evaluating preferences and are widely used. However, they are not suitable in all scenarios.

4 At What Point in the Medical Product Lifecycle is Patient Preference Information Most Useful, and for What Purpose?
The net present value of engaging patients in Patient Preferences in the Medical Product Lifecycle the MPLC is high in the early phases of development. The challenge is to secure the funding at this early stage of development.

5 Is There a Role for the Use of Preference Information Elicited from People Other than Patients to Inform the Medical Product Lifecycle?

Where the general public are the payers for care, their preferences might be relevant for informing priorities about health and social care provision, and resource allocation at a population level (and this is typically achieved, for example, through the use of the EQ-5D). Once resources are allocated to a specific area, patient preferences on how to develop or prioritise treatments or products within that area become most relevant.

6 How Might Patient Preference Information Best be Elicited from Patients and When?
For Example, is it Acceptable (or even Preferable) to Elicit Information Alongside Clinical Trials?
Whilst a trial may be helpful scientifically to accommodate randomisation and investigate comparative effects, it may not be the best setting for investigating preferences that would ideally be generalisable to the wider patient population.

Janet Wale, HTAi PCIG
E-mail: pcig.htai@gmail.com
