HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) E-Bulletin, September 2018

Enhanced quality and relevance of HTA through patient and citizen involvement

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

Welcome to the September E-Bulletin

Members of PCIG Steering Committee and its Working Groups are meeting in Stockholm at the beginning of October to advance our work programmes. The Working Groups are: Methods and Impact; Patient Involvement and Education; and Citizens and Community. This meeting also provides us with the opportunity to plan for HTAi 2019 Annual Meeting in Cologne, and review the resources and materials on the HTAi website. We look forward to reporting back on the meeting in the next E-Bulletin.

HTAi Matters

HTAi 2019 Annual Meeting
Deadlines for abstract submissions
Workshops and panels: 4 Oct, 2018
Oral, vignette and posters: 16 Nov, 2018

Deadline for travel grant applications: mid-November

HTAi Initiative For Public Health Outcomes Research And Measurement (INPHORM) IG survey
INPHORM IG is dedicated to developing and promoting appropriate HTA approaches, combining quantitative and qualitative evidence, expert knowledge, performance metrics and meaningful projections, to meet the needs of decision makers and to be applicable to diverse communities.
INPHORM has designed and developed a survey to find out how many HTA doers are evaluating or have public health interventions among the technologies they evaluate.
The aim of this survey is to identify the amount of time and analyses that organisations working on HTA spend when evaluating various public health technologies.
Click here to complete the survey.

What's Happening

IMI Stakeholder Forum 2018: 24 October 9am to 5pm, Brussels, Belgium

The Innovative Medicines Initiative (IMI) Forum registration is now open. This year we will be looking at IMI through the lens of cross-sector collaboration, and discussing the added value of technology convergence to address complex health challenges, especially in areas with huge public health need. Participation in this

event is free of charge but registration is obligatory. To register, simply fill in this form. By completing and sending this form, you agree and consent that your personal data provided in this form will be used, processed and shared with other organisations.

Webinar on artificial intelligence
https://www.kingsfund.org.uk/events/artificial-intelligence
The Kings Fund, UK is hosting a free webinar on 30 October about artificial intelligence in healthcare including issues for patients and citizens. Sign up at the link.

Submitted by Karen Facey

FDA Public Workshop: Partners in Progress 2018 - Cancer Patient Advocates and FDA 9am to 4pm, 27 November 2018, Silver Spring, MD
https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OCE/ucm600987.htm
This is the FDA Oncology Center of Excellence second annual educational workshop for new cancer patient advocates to provide basic training on the role of the FDA and cancer patient advocates in oncology product development.
Attendees will: Learn more about cancer treatment development, diagnosis, devices, drugs, and beyond; hear how patient advocates are crucial to supporting FDA's mission; hear about recent approvals; and interact with FDA staff and leadership.
Registration is available for both in-person attendance and remote webcast attendance.

Patient Voice Initiative workshops, Australia
Offered for patients or consumers interested in learning more about how decisions are made to fund medicines and treatments in Australia - and how your knowledge and personal experience can impact the decision-making process and help make a difference when it comes to access to medicines and treatments.
Kicking off in Sydney and Adelaide, the Patient Voice Initiative is holding a series of workshops across Australia - 'Influencing decisions about medicines and healthcare treatments'.
To register your interest for one of our first two events this year, click on the links below: - Sydney: 10.00am, Thursday 25 October 2018 - https://www.eventbrite.com.au/e/influencing-decisions-about-medicines-and-healthcare-treatments-registration-50466593941 - Adelaide: 10.00am, Monday 29 October 2018 - https://www.eventbrite.com.au/e/influencing-decisions-about-medicines-and-healthcare-treatments-registration-50521822130
Workshops in Brisbane, Hobart, Melbourne and Perth will be held in 2019. To register your interest in attending a workshop in one of these cities please email: contact@patientvoiceinitiative.org.au

Submitted by Ann Single:

From McMaster Health Forum
Understanding how to navigate the health system: A guide for citizens
Knowing how the Ontario health system is organized and how it works will better equip citizens and patients to navigate the system and also to be able to advocate for changes that would improve the system. A free online course, prepared by the McMaster Health Forum and supported by the Ontario SPOR SUPPORT Unit, is now available. The set of videos describes the 'building blocks' of Ontario's health system, as well as how those building blocks are used to provide care in the province. It will ensure citizens, patients and caregivers are better positioned to advocate for the changes they want to see in their system when opportunities arise.
This course is a companion to the course *Finding and using research evidence: A guide for citizens*. These courses are part of our 'Learn How' programs and complement the range of efforts we already take to engage citizens, including our citizen panel program.

Webinar. Understanding how to navigate the health system: A guide for citizens
This webinar describes the building blocks of Ontario's health system, as well as how those building blocks are used to provide care in the province. The material covered will ensure citizens, patients and caregivers are better positioned to advocate for the changes they want to see in their system when opportunities arise. There are four opportunities to attend this webinar. Register now for the webinar on:

- Thursday 11 October, 12:00pm to 1:00pm (English)
- Monday 29 October, 12:00pm to 1:00pm (English)
- Wednesday 14 November, 12:00pm to 1:00pm (English)
- Wednesday 28 November, 12:00pm to 1:00pm (French)

From the European Patients Forum
HTA: Improvements in Parliament but Still More Work to do
Over the summer there have been interesting developments on the Commission's legislative proposal for collaboration on Health Technology Assessment at EU level.
http://www.eu-patient.eu/News/News/hta-improvements-in-parliament-but-still-more-work-to-do/

Patient Engagement as a de-risking element of medicine development
The Nordic Life Science Days (NLS Days), the largest Nordic partnering conference dedicated to the life science industry, took place in Stockholm on 10-12 September - PARADIGM
Mathieu Boudes, coordinator of the IMI-PARADIGM project, chaired a session on patient engagement: what does it mean? The take-home message cannot be clearer: patient engagement is seen as a way to de-risk the development of medicines (short video from Boudes) in several ways.
http://www.eu-patient.eu/News/News/patient-engagement-as-a-de-risking-element-of-medicine-development/

EFNA working with Young Europeans with Neurological Conditions
The European Federation of Neurological Associations (EFNA) launched a survey on access and stigma among top concerns of young patients.
To best assess the issues that should be explored as part of this work, EFNA conducted a pan-European survey of 18 – 35-year olds living with neurological disorders. The survey also examined participants' feelings around levels of understanding of their disorder and their engagement in advocacy work or patient groups.
Between March 12th and May 15th 2018, 1368 responses to the survey were received from 39 countries. Almost 80% of the respondents were female, with the majority of the responses coming from the areas of Multiple Sclerosis, Myalgic Encephalomyelitis, chronic pain and migraine.
The top concern cited by respondents was 'access to medication/treatment', followed closely by 'isolation' and 'stigma'. This isolation and stigma could be attributed to the lack of public understanding of brain disorders.
You can read the complete survey report here: www.efna.net/efnayoungpeoplesurvey/
http://www.eu-patient.eu/News/News/an-introduction-to-efna/

Publications

The European Patients Academy on Therapeutic Innovation (EUPATI) Guidelines on Patient Involvement in Research in Development Articles
Last year we circulated the draft guidance from EUPATI on patient involvement in HTA for comment. The guidance draws on HTAi and HTA body guidance, the material in the EUPATI course and takes account of consultation comments received. The guidance itself was issued last year, but we are pleased to share with you the associated paper that has been recently published.
From Amy Hunter (Genetic Alliance), Karen Facey and Victoria Thomas (NICE)

Amy Hunter, Karen Facey, Victoria Thomas, David Haerry, Kay Warner, Ingrid Klingmann, Matthew May, Wolf See. EUPATI Guidance for Patient Involvement in Medicines Research and Development: Health Technology Assessment. Front. Med., 06 September 2018 | https://doi.org/10.3389/fmed.2018.00231
http://journal.frontiersin.org/article/10.3389/fmed.2018.00231/full?&utm_source=Email_to_authors_&utm_medium=Email&utm_content=T1_11.5e1_author&utm_campaign=Email_publication&field=&journalName=Frontiers_in_Medicine&id=345725

Submitted by Karen Facey

Evaluating facts and facting evaluations: On the fact-value relationship in HTA
Bjørn Hofmann, Ken Bond, Lars Sandman. Journal of Evaluation in Clinical Practice 2018;24(5):957-965.

Health technology assessment (HTA) is an evaluation of health technologies in terms of facts and evidence. However, the relationship between facts and values is still not clear in HTA. This is problematic in an era of "fake facts" and "truth production." Accordingly, the objective of this study is to clarify the relationship between facts and values in HTA. We start with the perspectives of the *traditional positivist account* of "evaluating facts" and the *social-constructivist account* of "facting values." Our analysis reveals diverse relationships between facts and a spectrum of values, ranging from basic human values, to the values of health professionals, and values of and in HTA, as well as for decision making. We argue for sensitivity to the relationship between facts and values on all levels of HTA, for being open and transparent about the values guiding the production of facts, and for a primacy for the values close to the principal goals of health care, ie, relieving suffering. We maintain that philosophy (in particular ethics) may have an important role in addressing the relationship between facts and values in HTA. Philosophy may help us to avoid fallacies of inferring values from facts; to disentangle the normative assumptions in the production or presentation of facts and to tease out implicit value judgements in HTA; to analyse evaluative argumentation relating to facts about technologies; to address conceptual issues of normative importance; and to promote reflection on HTA's own value system.

Impatient series #2
– what are companies looking for when choosing a rare disease
http://www.draccon.com/dracaena-report/2018/9/4/impatient-series-2-what-are-companies-looking-for-when-choosing-a-rare-disease

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The Path to Patient Centricity. Closing the 'How' Gap.
Findings from the 2nd Annual Aurora Project's Global Patient-Centric Benchmark Survey
Co-Leads: Lode Dewulf –Servier, John Elliott – Excellerate, Jack Whelan –Patient Advocate
https://excellerate.ca/the-path-to-patient-centricity-closing-the-how-gap/
https://www.ipsos.com/ipsos-mori/en-uk/pharmas-patient-focused-missions-more-important-ever
The Aurora Project is a volunteer group designed to bring the pharmaceutical industry together to focus on patient centricity within the industry. The survey was conceived in 2016 by eyeforpharma Chairman, Paul Simms, and Excellerate's Managing Director, Jill Donahue. The 2nd survey was conducted by Ipsos Healthcare between 14th July and 21st November 2017.
A total of 1,282 participants consisting of employees from biopharmaceutical and medical device companies (n=675), associated supplier companies (n=358), healthcare professionals (n=106) self-reported patients (n=70) and patient groups (n=73) completed the survey online; 113 countries were represented in the sample.
Themes that emerged from the data include:
How pharma sees itself, how patients see pharma. Across 10 metrics created by patients, patients consistently rated pharma companies less patient-centered than participants employed by industry.
Patient centricity goes hand in hand with engagement and pride. Measured for those employed as: a company making the world a better place (76%); people working with companies having pride in telling

people outside the pharma industry that they work in pharma, biotech/medical device company (81%); and whether their customers would say that 'I' help improve patient care (69%).
Trust in Pharma. 67% of those employed agreed that patients' trust would "slightly increase" or "significantly increase" if they secretly observed a typical day in their department. In a separate survey question, 36% of patients in the survey said they have "quite a bit" or "a lot" of trust in the pharmaceutical industry overall.
The link between patients' needs and business outcomes. 73% of patients agreed focussing on patients' needs leads to better business outcomes; 85% of those employed agreed.
Training is the missing ingredient to patient-centric execution. When asked about training or preparing people to behave in patient-focussed ways 53% of those employed by industry said: "We are actively looking for what and how to teach this to our people." Only 22% said: "We know exactly what and how to teach this to our people." Another 16% said: "We don't know what or how to teach this to our people."

From IMI PREFER
Drug Discovery Today _ September 2018
This study can inform different stakeholders on how to conduct, assess, and use patient preference studies and on when to include patient preference studies in development plans.

Factors and situations influencing the value of patient preference studies along the medical product lifecycle: a literature review. Eline van Overbeeke, Chiara Whichello, Rosanne Janssens, Jorien Veldwijk, Irina Cleemput, Steven Simoens, Juhaeri Juhaeri, Bennett Levitan, Jürgen Kübler, Esther de Bekker-Grob, Isabelle Huys. Industry, regulators, health technology assessment (HTA) bodies, and payers are exploring the use of patient preferences in their decision-making processes. 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. Factors and situations identified in 113 publications related to the organization, design, and conduct of studies, and to communication and use of results. Although current use of patient preferences is limited, we identified possible applications in discovery, clinical development, marketing authorization, HTA, and postmarketing phases.

Submitted by Nigel Cook

Compliance with requirement to report results on the EU Clinical Trials Register: cohort study and web resource. Ben Goldacre, Nicholas J DeVito, Carl Heneghan, Francis Irving, Seb Bacon, Jessica Fleminger, Helen Curtis. BMJ 2018; 362 doi: https://doi.org/10.1136/bmj.k3218 Published 12 September 2018
https://www.bmj.com/content/362/bmj.k3218

Europe's academics fail to report results for 90% of clinical trials
But nearly 70% of industry-sponsored trials report their results within a year of ending.
https://www.nature.com/articles/d41586-018-06676-8

Here's a short news-and-views piece about this topic:
https://www.the-scientist.com/news-opinion/about-half-of-clinical-trials-go-unreported-in-eu
Pharma companies are more compliant in posting results compared to universities
Sukanya Charuchandra, 14 September 2018
Approximately half of all clinical trials registered in the European Union have not reported results on the European Union Clinical Trials Register as required by the European Commission.
Goldacre and fellow researchers pushed out a tracker in early 2018 to record reporting compliance for US trials, finding that 59.1 percent of trials are compliant with US law and the US government could seek more than $600 million in penalties. However, "Compliance rates have not improved . . . over the six months since we started tracking".

Moving forward toward standardizing analysis of quality of life data in randomized cancer clinical trials
Andrew Bottomley, Madeline Pe, Jeff Sloan, et al. Cllinical Trials 2018. First published 24 August 2018.
http://journals.sagepub.com/doi/abs/10.1177/1740774518795637
https://doi.org/10.1177/1740774518795637
There is currently a lack of consensus on how health-related quality of life and other patient-reported outcome measures in cancer randomized clinical trials are analyzed and interpreted. This makes it difficult to compare results across randomized controlled trials (RCTs), synthesize scientific research, and use that evidence to inform product labeling, clinical guidelines, and health policy. The Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data for Cancer Clinical Trials (SISAQOL) Consortium aims to develop guidelines and recommendations to standardize analyses of patient-reported outcome data in cancer RCTs.
The Consortium will focus on three key priorities in the coming year: developing a taxonomy of research objectives, identifying appropriate statistical methods to analyze patient-reported outcome data, and determining best practices to evaluate and deal with missing data.
Consortium membership which includes regulators, patients, clinicians, and academics.

Statistical analysis of patient-reported outcome data in randomised controlled trials of locally advanced and metastatic breast cancer: a systematic review. M Pe, L Dorme, C Coens, E Basch, M Calvert, A Campbell et al. The Lancet Oncology 1 September 2018;19(9):459-469.
https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(18)30418-2/fulltext
Although patient-reported outcomes (PROs), such as health-related quality of life, are important endpoints in randomised controlled trials (RCTs), there is little consensus about the analysis, interpretation, and reporting of these data. We did a systematic review to assess the variability, quality, and standards of PRO data analyses in advanced breast cancer RCTs. We searched PubMed for English language articles published in peer-reviewed journals between Jan 1, 2001, and Oct 30, 2017. Eligible articles were those that reported PRO results from RCTs of adult patients with advanced breast cancer receiving anti-cancer treatments with reported sample sizes of at least 50 patients—66 RCTs met the selection criteria. Only eight (12%) RCTs reported a specific PRO research hypothesis. Heterogeneity in the statistical methods used to assess PRO data was observed, with a mixture of longitudinal and cross-sectional techniques. Not all articles addressed the problem of multiple testing. Fewer than half of RCTs (28 [42%]) reported the clinical significance of their findings. 48 (73%) did not report how missing data were handled. Our systematic review shows a need to improve standards in the analysis, interpretation, and reporting of PRO data in cancer RCTs. Lack of standardisation makes it difficult to draw robust conclusions and compare findings across trials. The Setting International Standards in the Analyzing Patient-Reported Outcomes and Quality of Life Data Consortium was set up to address this need and develop recommendations on the analysis of PRO data in RCTs.

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