NEWS

HTAi:
An International Society
for the Promotion of
Health Technology Assessment

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

Enhanced quality and relevance of HTA through patient and citizen involvement

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

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Welcome to the March E-Bulletin

And please remember to respond to Neil Bertelsen if you would like to be more active within the Interest Group at nb@neilbertelsen.com

We would appreciate you taking a few minutes to copy and paste your thoughts on the E-Bulletin and communicate them by email to socrates111@bigpond.com

I do/do not find the E-Bulletin informative and interesting to read
The information that is of **most** interest to me is .....
The information that is of **least** interest to me is ....
The E-Bulletin would be improved by....
Other suggestions I have are.....

HTAi Matters

HTAi 2018 Annual Meeting
https://www.htai2018.org/#
Join HTAi 2018 to engage with an extensive network of leaders, experts and key policymakers in lively discussions.

Any questions regarding Registration? Please contact **registration@htai.org**

Highlighted panel session: Patient Preference Data versus Patient Participation in HTA – Getting The Balance Right

Developing appropriate methodologies for incorporating new forms of evidence-based patient input into HTA decision-making will help agencies better understand the value of new treatments and enhance patient-centricity in resource allocation decisions.
The objectives of this panel session are to move the debate forward by:

- putting PPI in the spotlight and provoking debate around the advantages and disadvantages of traditional and newer forms of patient evidence

- providing attendees with the opportunity to examine and critique patient participation through engaging with PPI experts from across different sectors and viewpoints

Chair/Moderator: Eric Low – The Amyloidosis Research Consortium/Independent Healthcare Consultant
Initial debate: 'This house believes that patient preference data should replace patient participation in HTA appraisals' with Eric Low; Marion Danner - Institute of Health Economics · Cologne University Clinic.
Followed by audience vote and questions
Panel discussion: Kate Morgan – Myeloma Patients Europe; Rodolpho Hernandez – Aberdeen University; Marion Danner – Institute of Health Economics · Cologne University Clinic; Thomas Morel – UCB Pharma; Simon Fifer - CaPPRe
The development of Patient and Public Involvement (PPI) as a distinctive discipline of interest, represents a significant shift in recent years towards increased patient-centricity in health and resource allocation decisions. Patient input in health technology assessment (HTA) processes has traditionally involved written and oral evidence and involvement of 'patient experts'. More recently, systematic approaches to the incorporation of patient needs, choices, experiences, preferences and expertise are changing the nature of PPI, through inclusion of patient preference studies alongside established forms of patient input. The notion that patient preferences should be more systematically considered within HTA has emerged in part, as a response to evidence that demonstrates that the outcomes that have traditionally been measured in trials do not always accurately reflect what is important to patients. Studies that can present evidence of 'real-world' patient preferences may counter this imbalance and ensure greater patient-centricity in the assessment of new treatments. The increasing interest in patient preference elicitation studies has also responded to perceived challenges in traditional forms of patient input regarding its robustness, quality and consistency, and the perception that such input has a limited impact on the process. However, the role of patient preference data within future HTA contexts is a subject of continued interest and controversy: how can we embrace new forms of patient evidence alongside more traditional forms of input, while ensuring that the patient voice is not silenced by an increased reliance on empirical data forms?

PCIG Matters

The Patient Involvement and Education Working Group (PIE) Resource Directory: is starting to take shape and will be built into the new HTAi website to be launched in the next couple of months. We wish to thank Martyn Lee of Blue Mantis in the UK for his ready assistance and advice on WordPress requirements for the Directory https://www.bluemantis.com/

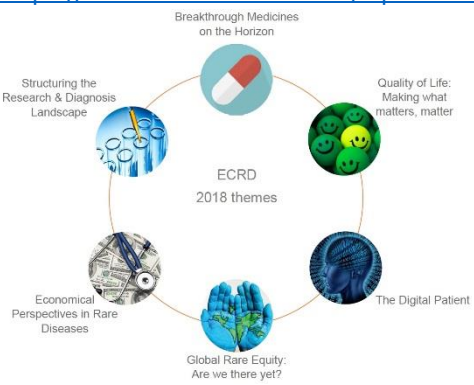
What's Happening

EUnetHTA 2018 Forum, Cologne 25 May 2018: Delivering products and services
https://www.eunethta.eu/about-eunethta/2018-eunethta-forum/

Rare Diseases

Eurordis and rare disease community welcomes proposal for future European cooperation on HTA Press release: https://www.eurordis.org/sites/default/files/PressReleaseEURORDIS_HTAProposal_Final..pdf

European Conference on Rare Diseases & Orphan Products, ECRD 2018 on 10-12 May, in Vie**nna, Austria**
https://www.rare-diseases.eu/wp-content/uploads/2018/02/ecrd2018themes.pdf#page=1



'Rare Diseases 360° – collaborative strategies to leave no-one behind' is the overarching theme. This theme reinforces the unique quality of this foremost event for the rare disease community in Europe, bringing together and facilitating effective policy discussions between all rare disease stakeholders, with the viewpoint of rare disease patients as equal experts.

Contributed by Marleen Kaatee

Patient and public involvement: Patient Representative for pCODR (CADTH) in Canada
Deadline for nominations is April 27th, 4 pm ET.
The successful candidate will have personal knowledge of, experience with, and understanding of issues related to cancer and its management (diagnosis, treatment, and care). They will also have a demonstrated understanding and appreciation of patient needs and priorities and an overall understanding of other patient issues and health care concerns that may impact cancer patient communities.
https://www.cadth.ca/sites/default/files/corporate/nomination/PERC/pERC_Patient_Member_Roles_Responsibilities.pdf

Contributed by Deb Maskens

PCORI Activities
PCORI accepts applications for is advisory panels on a rolling basis. Panelists bring voices from across the healthcare community into our work in a variety of ways. Among other duties, they refine and prioritize research questions for potential funding, and offer feedback on the design of clinical trials. A recent call ended 30 March. https://www.pcori.org/engagement/engage-us/pcoris-advisory-panels/advisory-panel-openings?

A PCORI video on helping clinicians in treating disease
Ellen Mowry, talks about how her PCORI-funded study aims to provide clarity for clinicians on which disease-modifying therapy should be prescribed for patients living with multiple sclerosis and whether they should be applied aggressively or not, based on evidence. https://vimeo.com/254039726?utm_source

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National Centre for Pharmacoeconomics (NCPE) Ireland: Public consultation
http://www.ncpe.ie/submission-process/public-consultation-2/
In 2016, the NCPE launched the Patient Organisation Submission of Evidence process, to supplement HTA reports with information collected directly by patients, from patients, detailing the real-life experience of living with the disease in question and how the new treatment may address the challenges arising from the disease. The NCPE have now undertaken a review of the process, and have developed new guidance documents, information sheets and templates to aid patient organisations through the submission process. We are holding a public consultation to give interested parties an opportunity to provide their feedback on these draft documents. Your input is essential, and we will assess all feedback received and use it to develop the finalised process and documentation. We welcome responses to all questions as well as any additional general comments you would like to make.
Your comments can be submitted by downloading and completing the consultation feedback form and emailing your completed form to us at info@ncpe.ie . The closing date for the consultation is 5pm on 30 April 2018.
Guidelines for Patient Organisations
Patient Involvement in HTA in NCPE-Overview
Patient Organisation Database Registration Form
Patient Organisation Submission of Evidence Template
Public consultation on Patient Organisation Submission Process
From: http://www.ipposi.ie/our-work/health-innovation/health-technology-assessment/

IAPO Global Patients Congress 24-26 May 2018, Florida USA

The congress strives to be a catalyst of cross-sector collaboration & innovation in patient empowerment through knowledge by bringing together top NGOS, academics, medicines regulatory bodies, researchers and many more. Register now: https://www.iapo.org.uk/registration

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An interview with IMI Programme Office, EUPATI project coordinator Jan Geissler and Matthias Gottwald of Bayer

On how European patients are benefitting from the work of EUPATI – a programme for patient involvement in medicines research and development, 20 March 2018

http://www.imi.europa.eu/projects-results/success-stories-projects/eupati-has-been-game-changer-empowerment-patients

With EUPATI we had the opportunity to implement a structured programme that trains patients and patient advocates on how drug development works and how all the regulatory processes around that work (JG). The toolbox on medicines R&D, the material that is available on the website and now used by more than 500 000 people worldwide, is a huge achievement and allows everyone around the world to use the high quality-controlled material we produced (MG).

We also established 18 national platforms which are based on the same model as EUPATI. The platforms are public-private partnerships led by patients, with academia and industry and in some countries also regulators. This has enabled patients on a national level to be involved in medicines R&D and regulatory affairs… The academic community benefited from the project by getting a neutral platform to collaborate with industry, regulators and patient organisations. Thanks to all the transparency rules and financial regulations in place, IMI has a very robust framework for this kind of collaboration to happen. What IMI has established is very unique on a global level. Without IMI, it would have been difficult to establish a similar project and fund it in a transparent and collaborative way without compromising the independence of any of the partners. From that perspective, IMI has been a key platform for this. (JG)

Project factsheet

National Institute for Health Research (NIHR) new set of national standards for public involvement in clinical research for organisations across the UK, March 2018

https://www.nihr.ac.uk/news/new-national-standards-launched-across-the-uk-to-improve-public-involvement-in-research/8141

The six standards aim to provide concise benchmarks for effective public involvement, alongside indicators against which improvement can be monitored, it said.

Developed through an 18-month partnership bringing together members of the public with representatives from the NIHR (England), the Chief Scientist Office (Scotland), Health and Care Research Wales and the Public Health Agency (Northern Ireland)

The partnership will now work with ten pilot sites across the UK as they put the standards to practical use in their own working environment, but is also encouraging "as many groups and organisations as possible" to employ the new standards and share learning and experiences.

Involvement in governance and leadership is interesting as the sixth standard, after inclusive opportunities, working together, support and learning, communication, impact.

McMaster Health Forum Finding and using research evidence: A webinar for citizens

Knowing how to find and use the best available research evidence will better equip citizens and patients to make informed decisions about their health, and to effectively advocate for health system changes. This webinar describes the most commonly cited frustrations people have when trying to access research evidence, and provides solutions to overcome these frustrations. The McMaster Health Forum is pleased to provide three opportunities to attend this webinar. Register now to join us on:

- Wednesday 18 April, 2:30pm to 3:30pm (English)
- Wednesday 16 May, 12:00pm to 1:00pm (French)
- Wednesday 23 May, 12:00pm to 1:00pm (English)

https://www.mcmasterforum.org/learn-how/public-events/event-item/finding-and-using-research-evidence-a-guide-for-citizens-apr18

ISPOR training in PRO Measures: Development and Use in Clinical Research, 10-11 April, Durham, USA
https://www.ispor.org/Event/Index/2018PRODurham
Patients, providers, payers, and regulators increasingly demand evidence signifying the real differences new health care interventions make to people's lives. Patient-reported outcome (PRO) data is critical for demonstrating the impact of interventions on how a patient feels and functions.
Patient-reported outcomes (e.g., fatigue, pain, physical functioning, social functioning) can provide great value to research, but can present significant challenges. Do you need to know how to conceptualize, develop, and evaluate PROs so you can effectively incorporate them into your clinical research? Enjoy a thorough deep-dive into the different types and suitability of measures, methods for developing new measures, and best practices for collecting and analyzing PROs in clinical trials. This training includes a friendly introduction to the technical aspects of Classical Test Theory and Item Response Theory for the evaluation of the psychometric properties of a PRO measure and incorporates the latest recommendations from the FDA and other regulatory bodies in the design and application of PRO measures. After the program, you'll be able to take advantage of the great value PROs brings to research as well as understand how to overcome its significant challenges.

DIA Measuring Impact in Patient Centered Drug Development conference October 2-3, North Bethesda, USA http://www.diaglobal.org/en/conference-listing/meetings/2018/10/measuring-impact-in-patient-centered-drug-development?
Never before has there been such momentum for engaging patients in the development of medical products, not just in clinical trials but throughout the medicines lifecycle. Impact measurements can provide key information to determine whether engagement efforts are working, stakeholders' efforts have been well-spent, and that patients are benefiting. Yet recent work has shown that companies have not consistently defined or collected metrics to assess the impact of their patient-centric efforts

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From European Patients Forum (EPF) Newsletter
In our blog, EPF member EFCCA – the European Federation of Crohn's and Ulcerative Colitis Associations – presents the findings from its project 'Mapping of innovative medicines and devices in EFCCA member countries' in which 31 countries participated. http://www.eu-patient.org/blog/?p=798#more-798

Mathieu Boudes has been announced as PARADIGM Coordinator: - on patient engagement during the lifecycle of medicines http://imi-paradigm.eu/

Information on biosimilars:
http://www.ema.europa.eu/docs/en_GB/document_library/Leaflet/2017/05/WC500226648.pdf
Questions and Answers for patients http://ec.europa.eu/DocsRoom/documents/26643

AllTrials Campaign
I hope you've seen our new tool, the FDAAA TrialsTracker, which identifies every individual clinical trial that fails to report its results, live online. Now everyone can see the individual institutes, companies and researchers responsible for unreported trials, the very day they go overdue. Three weeks ago we highlighted just one of these overdue trials, from researchers at Columbia University. It was a trial on pain relief in labour: a real world issue that really matters. Just one week after we shone our spotlight, the researchers submitted their results.The AllTrials campaign could now start tracking down and identifying more missing trials - those from before this month, on the medicines we are currently using - and we think it's urgent, because with every day that passes, software and individuals retire, and we'll lose the possibility of getting those results reported.

From now on, every week, we will highlight in a piece in the BMJ a trial that is overdue to report results as identified by the FDAAA Compliance Tracker. http://fdaaa.trialstracker.net/

Publications

Decision making in NICE single technological appraisals: How does NICE incorporate patient perspectives? F Hashem et al. Health Expectations 2018;21(1):128-37.
The National Institute for Health and Care Excellence (NICE) has an explicit mandate to include patient and public involvement in the appraisal of medicines to be available for funding on the NHS. NICE involves an appraisal committee who are required to take on board experiential evidence from patient experts alongside population-based evidence on clinical and cost-effectiveness when making a decision whether to fund a drug. This paper considers how NICE Single Technological Appraisal (STA) committees attempt to incorporate the views of patients in making decisions about funding medicines on the NHS.
Three data collection methods were used: analysis of documentary evidence sent by NICE, non-participant unstructured observations of the open and closed sessions of meetings and qualitative interviews.
Our analysis showed how the committees displayed a preference for an ideal-type of patient representative, disagreement among the committee when weighing-up patient statements in the STA process and more pre-preparation support for patient involvement.

Health democracy in Europe: Cancer patient organization participation in health policy. K Souliotis et al. Health Expectations 2018;21(2):474-84

Optimizing patient and public involvement (PPI): Identifying its "essential" and "desirable" principles using a systematic review and modified Delphi methodology. RL Baines et al. Health Expectations 2018;21(1):327-35.

Experience-Based Values: A Framework for Classifying Different Types of Experience in Health Valuation Research. Patricia Cubi-Molla, Koonal Shah, K Burstrom. The Patient - Patient-Centered Outcomes Research (2018). Whether health values should be elicited from the perspective of patients or the general public is still an open debate. The overall aim of this paper is to increase knowledge on the role of experience https://link.springer.com/article/10.1007/s40271-017-0292-2

Engagement of Canadian Patients with Rare Diseases and Their Families in the Lifecycle of Therapy: A Qualitative Study. Andrea Young, Devidas Menon, Jackie Street, Walla Al-Hertani, Tania Stafinski. The Patient - Patient-Centered Outcomes Research (2018). Patient involvement is increasingly recognized as critical to the development, introduction and use (i.e. the lifecycle) of new and effective therapies, particularly those for rare diseases https://link.springer.com/article/10.1007/s40271-017-0293-1

Beyond Adoption: A New Framework for Theorizing and Evaluating Nonadoption, Abandonment, and Challenges to the Scale-Up, Spread, and Sustainability of Health and Care Technologies. T Greenhalgh et al. J Med Internet Res. 2017 Nov; 19(11): e367. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5688245/
Development of a nonadoption, abandonment, scale-up, spread, and sustainability (NASSS) framework including questions in 7 domains: the condition or illness, the technology, the value proposition, the adopter system (comprising professional staff, patient, and lay caregivers), the organization(s), the wider (institutional and societal) context, and the interaction and mutual adaptation between all these domains over time. Subject to further empirical testing, NASSS could be applied across a range of technological innovations in health and social care. It has several potential uses: (1) to inform the design of a new technology; (2) to identify technological solutions that (perhaps despite policy or industry enthusiasm) have a limited chance of achieving large-scale, sustained adoption; (3) to plan the implementation, scale-up, or rollout of a technology program; and (4) to explain and learn from program failures.

What do we mean by informed health choice? Blog by Carl Heneghan, 30 January 2018
http://blogs.bmj.com/bmjebmspotlight/2018/01/30/mean-informed-health-choice/?utm_content=buffer627fc&utm_medium=social&utm_source=twitter.com&utm_campaign=buffer
...we need a generational change in how patients and the public equip themselves to be informed. Effective healthcare practitioners will need to develop and maintain the skills needed to share decisions effectively....

Chalmers I, Oxman AD, Austvoll-Dahlgren A, et al. Key Concepts for Informed Health Choices: a framework for helping people learn how to assess treatment claims and make informed choices. BMJ Evidence-Based Medicine 2018;23:29-33.Open access
Testing Treatments interactive: http://en.testingtreatments.org/key-concepts-for-assessing-claims-about-treatment-effects/

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