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

The Patient and Citizen Involvement interest group had a very successful series of events in Vancouver. Thank you to all those who were able to attend and make this such an important and insightful meeting for us all. We kicked off the activities with a full-day workshop looking at how we lighten the load for patients and patient organisations that input into HTA processes. This led to four separate areas that could help reduce the burden; a full workshop report is being written up at the moment and will be distributed to you all. We were also pleased to see so many panel sessions on patient involvement covering every aspect of the HTA process from horizon scanning, early dialogues and the assessment itself, which shows just how much patient involvement is being embedded into HTA processes at some (but still not all) HTA agencies. There is still a lot of work to do to embed patient involvement more fully into HTA systems and to help us do this, we need active involvement from those of us that are committed to this.

With that in mind, we held a special session as part of our annual business meeting where people were invited to visit stations manned by people from our three working groups to learn more about their work and to sign up to be involved in the various work streams and projects. This was a great success, so thank you to those who signed up! Obviously, for those of you that could not attend Vancouver, we are still looking for active participation in our work streams and I invite you all to write in to find out how you could be involved too.

Neil Bertelsen, Chair

HTAi Matters

Governance

Visit the new HTAi website with its vastly improved layout and accessibility. Though we do have some work to do updating it. https://htai.org/interest-groups/pcig/

New addition to Steering Committee

Todd Stephenson of Johnson & Johnson in Australia is now part of our Steering Committee, co-opted to look at funding. He joins Neil Bertelsen (Chair, Germany), Janney Wale (Previous Chair, Australia), Ken Bond (Canada), Sophie Staniszewska (England), Heidi Livingstone (England), Hervé Nabarette (France), Valentina Strammiello (Belgium), Thomas Morel (Belgium), Sally Wortley (Australia) and Karen Facey (Scotland) with Rebecca Addo as our Technical Officer.

Seven were in Vancouver, and most were at the pre-conference workshop, co-facilitated by Neil Bertelsen and Eric Low with Anke Holtorf and Rebecca Addo ensuring that the day ran smoothly and was well captured..

A PARADIGM scoping meeting with HTA agencies was held in Vancouver, helping to move this project forward and to make plans for required activities.

HTAi Annual Meeting, June 2018 in Vancouver
The full 2018 Annual Meeting programme is available still at: https://www.htai2018.org/full-programme/
I think I can safely say that all of us who were in Vancouver had an excellent conference and enjoyed ourselves greatly. The hospitality shown to us made us very relaxed and happy despite our busy-ness.

The overall theme this year was 'Strengthening the Evidence-to-Action Connection' and, for the first time, the meeting was a 'Patients Included' event. We in PCIG will work to ensure that all future events have this designation.
The title of the PCIG workshop was 'Building a shared resource of patient experience and preferences to improve HTA' and led to lively discussions. A summary is being prepared. Similarly the PCIG Annual Business Meeting was energised and we thank the people who have expressed an interest in contributing to our Working Groups. Enjoy your summer and we look forward to working with you.
As always it was often very difficult to prioritise where you should be with the parallel sessions. All were well attended and the quality of speakers was high. One thing I did note was that 'disinvestment' of technologies is now frequently referred to, much more politely, as 'reassessment'.
Patient Involvement and Education Working Group welcomes Sarah Berglas, Patient Engagement Officer at CADTH to the group, as well as 7 new members since Vancouver. This extends the countries we cover to nine.
Our panel session "Innovative Methods for Patient Evidence in a World of Real-World Evidence" went well and Emil Chiauzzi of PatientsLikeMe (PLM) is a very pleasant well informed person who was great to work with. It is very interesting to see the depth of information PLM can capture from people and the variety of ways that information is used including for the FDA and quality measures as well as citizen driven research.

One item that was brought to my attention was that our paper on patient involvement in HTA was in the top 10 accessed articles in IJTAHC (Wale J, Scott A, Hofmann B, Garner S, Low E, Sansom L. 2017. Why patients should be involved in Health Technology Assessment. International Journal of Technology Assessment in Health Care, 1-4. doi:10.1017/S0266462317000241).

Why Patients Should be Involved in HTA
1. Patient's Rights Perspective
    - Alma Ata Declaration (1978) – "…people have the right and duty to participate individually and collectively in the planning and delivery of their health care"
    - WHO resolution on health intervention and HTA in support of universal health coverage
2. Need for Value Determination
    - Value determinations and value judgements needed beyond cost effectiveness
3. Evidentiary Contributions
    - Patients provide the 'lived experience' that reflects benefits (and harms) to the patient that may be broader that the outcomes reflected in trials or traditional quality of life data
4. Methodological Perspective
    - New approaches for obtaining timely evidence
    - Patient input on design and relevant endpoints for clinical studies

Not only that, when I was looking at the panel presentations from ISPOR 2018 in Baltimore I saw it referred to by Deborah Marshall of University of Calgary in Canada, in IP4: Incorporating quantitative patient

preference information into healthcare decision-making processes: is HTA falling behind?
https://www.ispor.org/Event/ReleasedPresentations/2018Baltimore

Mark Skinner was in Vancouver to present on his collaborative project, which I asked him to write about:
Core outcome set for gene therapy in haemophilia: Results of the coreHEM multistakeholder project
coreHEM, a multi-stakeholder project to define a core outcome set for clinical trials of gene therapy in hemophilia, has announced publication of coreHEM results. This core set will ensure that patient perspectives on critical outcomes are included in pivotal trials, allow fair comparisons between alternative treatments, and allow more accurate assessments of the value of these therapies. Participating experts and stakeholders included patients, clinicians, payers, health technology assessment groups, regulators, life sciences companies and others. The results were published in *Haemophilia* 20 May 2018. The final report, "coreHEM: Developing Comparative Effectiveness Outcomes for Gene Therapy in Hemophilia," details the methods, results, and impact identified by the initiative, as well as the final core outcome set, and preliminary work on measurements and instruments identified for those outcomes. The coreHEM project was jointly led by the National Hemophilia Foundation (NHF), the Green Park Collaborative (GPC), and McMaster University. The coreHEM project is an example of a multi-stakeholder effort including patients that may have interest and value to other disease groups.

If you want to discuss this project with him his email is mskinnerdc@gmail.com

What's Happening

PARADIGM – please complete our survey
Short survey to Find Out Stakeholders' Needs and Expectations for Patient Engagement in the Medicines Research and Development Lifecycle
https://imi-paradigm.eu/survey/

Take the survey and don't forget to share the link with your network!

Legislative proposal for EU collaboration on HTA
'The way forward for HTA cooperation in the EU – the views of stakeholders', 9 July in Brussels
https://ec.europa.eu/health/technology_assessment/events/ev_20180709_en
Session 1 – Engaging with patients and clinicians on HTA: Current status and future direction
A scene-setting presentation by a representative of the patient community, followed by a multi-stakeholder panel discussion. Involvement of the patients and health care providers in the joint work, in particular in the joint clinical assessments, the joint scientific consultation as well the broader strategic work, such as developing the work programmes of the Coordination Group, identifying emerging technologies, and ensuring that the outcomes and factors relevant to patients are considered. [EU patient representative; HTA agencies with engagement processes in place or being developed; Healthcare provider representative]
Session 2- Generating evidence that meets the needs of patients and health system decision makers
A scene-setting presentation by a representative from an HTA body, followed by a multi-stakeholder panel discussion. The discussion on the pre-market phase will have a specific focus on the joint scientific consultation and how it can support the generation of evidence that meets the needs of HTA bodies. [Representatives from two HTA bodies; Industry representative; EMA representative; Patient representative]
Session 3 - Managing uncertainty in the post-launch phase
A scene setting presentation by the healthcare provider representative, followed by a multi-stakeholder panel discussion. Post-launch evidence generation space and the potential for updates of joint clinical assessments using newly generated evidence. [Payer representative; Healthcare provider representative; Representative from an HTA body; Industry representatives]

Web streaming: The meeting will be broadcast live from 10 AM to 6 PM.
Registration to the event is available here. You should be registered in the European Commission Access System – EULogin.

Submitted by Marleen Kaatee
NOTE: Marleen will be attending and proposes early dinner on Sunday July 8 for those other PCIG members in town.

The ECPC response on the legislative proposal of the EU Commission for a regulation on HTA
http://ecpc.org/ECPC%20Response%20to%20HTA%20-%20Policy%20Paper.pdf

On 15 May, the EU Health Commissioner, Vytenis Andriukaitis, convened a meeting of the patients and consumers pool participating in the Commission HTA Stakeholders pool (the other member pools are those of healthcare providers, industry, payers) to discuss the future of the Commission's proposal.

Nine colleagues and I, representing large European umbrella organizations, participated in this meeting and we had a debate on the basic provisions of the legislative proposal. In view of that meeting ECPC prepared its response, asking for meaningful patient involvement in HTA, including participation in the Coordinating group. There is a meeting in Brussels on July 9 to debate the Commission proposal.

Kathi Apostolidis

European Patients Forum June Newsletter
In its position paper (May 2018) on the European Commission's Proposal for a Regulation on Health Technology Assessment, EPF calls on EU Member States to put patients first and make progress on HTA negotiations http://www.eu-patient.eu/globalassets/policy/hta/epf-position-statement-on-hta.pdf
The European Patients' Forum supports the Commission's legislative proposal. We call for certain aspects of the proposal to be clarified and/or strengthened. We believe this will help ensure the adoption of a Regulation that will bring real progress in advancing equitable access to high-value medical technologies across the EU. As the European cross-disease patient umbrella organisation, EPF is committed to working closely with the European legislators and in dialogue with our membership towards a sustainable European framework for HTA where patients are actively involved as partners and which ensures timely and equitable access for patients to medical technologies that add value and improve their lives

The European Commission's proposal on HTA was discussed at the Employment, Social Policy, Health and Consumer Affairs Council (Social policy and health), 21-22/06/2018
In the course of the EPSCO Council meeting divergences of views among Member States emerged. Member States with more advanced HTA systems – such as France and Germany – tend to be wary of the proposal mainly because of the foreseen mandatory uptake of joint clinical assessments, while smaller Member States and those with HTA systems in a more formative stage – including Belgium, Romania and the Baltic countries – tend to support the legislative proposal and the mandatory uptake requirement.
EPF believes that without ensuring uptake of joint work in national assessments, there will be no progress or added value for public health, beyond what is already achieved through voluntary collaboration in EUnetHTA. We also believe that through a constructive dialogue it will be possible to agree on a solution that both ensures the integration of joint assessments in national assessments without duplicating, and still gives each Member State the possibility to address their national specificities.
The Council eventually concluded that Germany and France will start to draft an alternative proposal for EU-level HTA, which does not include mandatory uptake. The European Parliament, meanwhile, is developing its own position on the HTA proposal. The Austrian Presidency has committed to take forward the topic to facilitate some additional constructive debates to overcome the most challenging aspects embedded in the proposal.

Kaisa Immonen and Valentina Strammiello

The EUnetHTA procedure for Early Dialogues (ED) for medical devices and the related briefing book template - now open for public consultation through July 20, 2018.
https://www.eunethta.eu/public-consultation-early-dialogues-for-medical-device-developers/
Following on the successful launch of the JA3 procedures for European Network for Health Technology Assessment (EUnetHTA) Early Dialogues (ED) for Pharmaceutical products, EUnetHTA is pleased to announce the future launch of EDs for Medical Devices.
This initiative builds on previous pilots on early dialogues for medical devices including EUnetHTA JA2 and the SEED project financed by the European Commission.
The main benefits of the ED procedure include: a streamlined procedure for applicants; increased mutual understanding and problem-solving ability between HTA bodies through a structured interaction; improved coordination with, and greater participation of HTA bodies in EDs through a unique contact point and a Scientific Coordinator; and the centralised recruitment of HTA bodies.
Patient representatives and healthcare professionals may also participate in the parallel consultation procedure on a routine basis so that their views and experiences are incorporated into discussions.
Please note that the procedure may evolve based on feedback received from participants (internal and external stakeholders) via a post-ED questionnaire.

Submitted by Marleen Kaatee

Public involvement in Scotland
Scotland has been exploring how it can involve the general public in its health policy decision-making over the past decade. Last year it held a number of multistakeholder meetings to discuss specific issues about public involvement to ensure person centred care. These are reported at the following link; and those under item 4 about priority setting are of particular relevance to HTA.
http://www.aog.ed.ac.uk/research/health_policy_forum

Submitted by Karen Facey

ISPOR released presentations May 2018, Baltimore
https://www.ispor.org/Event/ReleasedPresentations/2018Baltimore

These include the 3rd plenary session: "Examining the role of patient preferences to inform regulatory decisions" where Jeff Shuren gives the FDA perspective for devices and radiological health [Presentation]
Issue Panel Presentations including IP4: "Incorporating quantitative patient preference information into healthcare decision-making processes: is HTA falling behind?" [Presentation]

Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders
FDA draft guidance
http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

This guidance document is being distributed for comment.
The purpose of Guidance 1 is to present sampling methods for collecting information on the patient experience that is representative of the intended population to inform the development and evaluation of medical products throughout the medical product lifecycle. In addition, this document discusses methods on how to operationalize and standardize the collection, analysis, and dissemination of patient experience data.

Submitted by Nigel Cook

PREFER communications update: New project videos

You can find the short plain language clips on the project start page http://www.imi-prefer.eu/ in a video slider starting with one about the why we benefit form a public-private partnership (new), followed by one about what we do, one about what a patient preference is, and ending with one about whose preferences we are looking at.

You will find all our videos on the PREFER YouTube channel https://www.youtube.com/channel/UC7LCAR83VK2fFAMmTG05kkg where we have also posted a video from the April 10 webinar with Brett Hauber and Stephanie Christopher on using the threshold technique to elicit preferences for Parkinson's Disease device attributes.

You can view all the videos about the project in one go (total viewing time 5 minutes 44 seconds) in our Learn about PREFER playlist https://bit.ly/2ywsFXW and find direct links to all the individual clips below.

PREFER was mentioned in a guest column by Barbara Lopez Kunz (global chief executive, DIA) in Clinical Leader recently: https://www.clinicalleader.com/doc/what-s-next-in-patient-engagement-0001

Links to all videos:

- Giving patients a voice in drug development: https://www.youtube.com/watch?v=4y82jLvW7RA
- Why we need a Public-Private partnership: https://www.youtube.com/watch?v=JqREBRWTCTk
- What is a patient preference: https://www.youtube.com/watch?v=fZenp_IWsPo
- Whose preferences are we collecting: https://www.youtube.com/watch?v=P_4BMtEWOMo
- Webinar: Eliciting Patients' Preferences for Parkinson's Devices: https://www.youtube.com/watch?v=wSRXMxgUOeM&t=147s

Submitted by Nigel Cook

McMaster Health Forum Update June 2018 - Sharing our expertise internationally

We recently had the opportunity to share ideas and best practices regarding public and patient engagement with our Colombian colleagues at Colombia's Instituto de Evaluación Tecnológica en Salud (IETS) during a week-long workshop (led by François-Pierre Gauvin, the Forum's Senior Scientific Lead, Citizen Engagement and Evidence Curation) and a one-day symposium to celebrate the five-year anniversary of IETS. The discussions, which engaged the Colombian Minister of Health and other health-system leaders, included great exchanges on how to improve health technology assessment and make fair and sustainable coverage decisions.

New Cochrane Co-Chair

Cochrane's Governing Board appoints Marguerite Koster as Co-Chair, from September 2018. Marguerite is a Senior Manager at Kaiser Permanente, US, joining Martin Burton, Director of Cochrane UK as the other Co-Chair.

ISQua 2018 Kuala Lumpur 23 to 26 September

Workshop: Patient Reported Outcomes 101 – Principles, Tools and Implementation: A Stepwise Practical Approach. Speakers: Eugene Nelson, Brant Oliver, Eyal Zimlichman.

Using patient reported outcomes (PROs) to bring the voice of the patient into the heart of healthcare delivery has been shown to improve patient-clinician partnerships, health outcomes, healthcare value and patient-centered outcomes research. The question is how to harness the potential of using PROs by building them into the flow of care and into clinical information systems to support person-centered care, improvement and research. We will feature real world cases and practical methods for building PROs into busy clinical settings.

Submitted by Russell McGowan

Publications

Human nature: the next frontier for pharma?
By Jim O'Donoghue, 29th June 2018
With the rise of digital health and the inexorable fall in drug development returns on investment, there is no denying that the pharma industry is at a crossroads.
In recent years, firms have invested in Patient Support Programmes (PSPs) to demonstrate added-value and gather real-world evidence to help address payers' increasing concerns over the high cost of new drugs. However, many in the business now understand they will need to take the plunge into the mostly unchartered territory of behavioural science if they want to truly influence patient outcomes.
Leading companies including Novartis, AstraZeneca, Sanofi and Takeda are aware they need to work out how to deliver patient support programmes which are highly personalised to individual patient needs, according to a recent survey we carried out with eyeforpharma.
But it won't be without its difficulties. This route takes companies towards more holistic healthcare, focused not just on the pharmaceutical therapy but on the patient as a whole and their engagement with their condition - far out of the traditional comfort zone of many pharma companies.
This step into the unknown requires an uncomfortable shift in brands' own behavioural habits by challenging a traditionally risk-averse sector to embrace an agile and entrepreneurial approach and to explore new business models...
Our survey of 20 top global pharma insiders uncovered widespread concerns about the effectiveness of traditional patient support programs (PSPs) currently used by the industry. Typically involving nurse-led call centres, using first-generation tech like texts, alerts and emails, delivered in a standardised way for patient populations, they often fail to deliver the improved patient outcomes, cost-effectiveness and scale that payers and regulators increasingly expect to see.
There is general agreement across the industry that the next generation of PSPs will need to be rooted in behavioural science and recognise that patient needs in relation to the management of their conditions will change over time. However, it is clear there is no industry consensus on what this transformation will look like...
By marrying technology and behavioural science, we can deliver truly personalised, intelligent PSPs that respond to individuals' everyday routines and encourage adherence by appealing to their personal goals and drivers. For example, we have developed sophisticated new platforms that allow patients and their caregivers – clinicians, family, peers – to create plans for how they manage their treatment and condition based on shared information.
This shift is going to require a willingness by the industry to go beyond the brand and reorganise themselves around the needs of patients. A brand-based approach worked when the objective was narrowly focused only on supporting patient access to a drug and reimbursement. But, with most conditions treated by more than one medication, and patients increasingly having multiple conditions, single-brand solutions are just too limited...
http://www.pharmatimes.com/web_exclusives/human_nature_the_next_frontier_for_pharma_1242457

Everyone can become a researcher! 5 April, 2018
By Tom H van de Belt
https://www.linkedin.com/pulse/everyone-can-become-researcher-tom-h-van-de-belt-phd/

Traditionally, 'patient participation' in scientific research was mostly limited to being a 'study subject'. This is a pity, since patients are - as described by Ian Kennedy - the 'experts' in their own field, or 'the experts in having a disease', with expertise that is impossible to obtain for researchers. Moreover, they are often the end-users of new therapies or technologies. The benefits of active patient participation in research have been well described and the leading British Medical Journal requires researchers to describe how patients are involved during various research stages. In this article we proudly present "REach", the tool that allows

anyone (patients, patient organizations, physicians and nurses), to easily set up an observational study and collect rich 'real life' data.
We are in the middle of the digital age. Billions of people use their smartphone to communicate and exchange information. Interestingly, an increasing number of people collect health data on their smartphone such as information about their mood, activity level, nutrition or vital signs including blood pressure or blood glucose levels. Medical research could greatly benefit from these 'real life' data, particularly since participation rates in observational studies have been declining for the last two decades. People may be too busy or do not feel the need to fill out questionnaires, self-measure, or complete lifestyle diaries. The challenge is, however, how to make these data available for scientific studies, in a simple way, but with the user in control.
Together with patients, doctors and members of a medical ethical committee, we explored this new concept in which 1) patients could actively participate in research such as by setting up their own study and 2) study participants could easily share their data with researchers. First, we emphasized that patients, since they collect and own data, they should also be in control. This resulted in the 'Personalized Consent Flow' that can be used by both study participants and researchers to decide what data will be shared, for what purposes, and for how long. Users can select 'modules' to eventually build a study, including an informed consent procedure, open and multiple choice questions, active tasks and health data sharing. After creating the study, a smartphone app is created and can be downloaded from the app store. T Then, we selected Apple's iOS as the preferred platform since Apple's ResearchKit is highly secured and allows app developers not only to create questionnaires and extract health data, but also includes a variety of validated tests such as hearing and movement tests. The challenge was - as the director of REshape Lucien Engelen already recognized in 2016 - how to create a user friendly 'drag and drop' interface that could be used by lay people and not by 'coding experts' only.
EA Rake et al. Personalized Consent Flow in Contemporary Data Sharing for Medical Research: A Viewpoint BioMed Research International 2017, Article ID 7147212
MMHJ van Gelder, LJLPG Engelen,T Sondag, Tom H van de Belt. Utilizing Consumer Technology (Apple's ResearchKit) for Medical Studies by Patients and Researchers: Proof of Concept of the Novel Platform Reach. Journal of Participatory Medicine 2018; 10(2): Apr-Jun

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What do stakeholders expect from patient engagement: Are these expectations being met?
M Boudes' P Robinson, N Bertelsen, N Brooke et al. Health Expectations 2018
This paper addresses patient engagement in medicine development and during the life cycle of a product. A survey was conducted through 59 interviews. HTA was grouped with payers and purchasers. The paper concludes that structure and guidance are needed for patient engagement. Open access.

Achieving successful community engagement: a rapid realist review
E De Weger et al. BMC Health Services Research 2018;18:285.
Community engagement is increasingly vital to achieving high quality, efficient and collaborative care - but health and care organisations are still searching for the most effective ways to engage. This review discusses barriers and enablers for engaging communities in the planning, designing, and/or delivering of health and care services.
https://bmchealthservres.biomedcentral.com/articles/10.1186/s12913-018-3090-1

Submitted by Karen Facey

How better reporting can improve patient engagement practice
A new systematic review published in Research Involvement and Engagement finds that less than 1% of published clinical trials contained critical information on patients engaged in the research.
http://blogs.biomedcentral.com/on-medicine/2018/05/22/how-better-reporting-can-improve-patient-engagement-practice/

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