HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG)
E-Bulletin, May 2016

Enhanced quality and relevance of HTA through patient and citizen involvement

Welcome to this month's E-Bulletin

Unlocking the Value Potential of New Technologies in Health Care Waste in Science
New Ways of Measuring Value - **Global Experiences in Universal Health Coverage**

The PCIG had its Annual Business Meeting during the HTAi 2016 Annual Meeting in Tokyo. At this meeting Janney Wale handed the Chair position over to Neil Bertelsen. Our new Terms of Reference that incorporate a permanent Vice-Chair and the Patient Panel were presented; as well as the Register of Interests for Steering Committee, Working Group and Patient Panel members to complete. It is important that people from a range of background interests are involved. Janney remains active and on the Steering Committee as past Chair, and wishes to thank everyone for their kind thoughtful words at handover.

Our preconference workshop 'East meets West: what we can learn from each other about patient perspectives and adding value to HTA and policy decision making' was well attended. The workshop was co-facilitated by Neil and Janney. It gave an excellent opportunity for people to talk about their experiences of patient and public involvement in HTA in a range of countries, linked by responses to a questionnaire that had been sent to HTAi Patient Panel members and others involved in patient involvement in March/April of this year. People who attended commented that there was good energy in the room, people were very willing to share experiences, and that everyone wants to 'make a difference'. People's hopes and expectations of the day were broadly met. The broad involvement in the workshop meant that people met each other and formed friendships right at the beginning of the conference.

We were very fortunate to have instantaneous translation for this workshop. We thank EFPIA Japan for ensuring that this happened and that language was not a barrier to participation. Bruno Rossi and patient advocate Naomi Sakurai, both from Japan, will prepare a summary of the workshop in Japanese and send it to Japanese patient groups that did not attend.

Patient values and experiences, patient and public involvement in HTA and policy decision making were present through the entire conference, from East to West, and so we were kept busy. It was also good to have some of our HTAi Patient Panel present to share our activities and provide vital contributions.

The Annual Meeting did provide an opportunity to consider:

- The role of HTA in universal health coverage
- The rationale for political adjustment in decisions
- The dilemma of cost-saving versus investing for innovation
- Scientific evidence versus pragmatic decision making
- The utilization of information technology in HTA
- The role of patient participation in decision making
- Cultural differences (e.g., utility measurement, expectations to the government, societal consensus).
- Country-specific HTA
- International data sharing and evidence transferability

International Clinical Trials Day with BioMed Central 20 May

Eric Low of Myeloma UK and a member of our HTA Patient Panel put together a blog on 'Logistical planning for trial delivery and data management' as part of a series for the 2016 International Clinical Trials Day. Individual blogs were released in the week leading up to the day, covering the different stages conducting a clinical trial, its design and management.

Thanks to Eric and Louise Farmer of Myelona UK,Ella Flemyng (who was in Tokyo) and Sophie Marchant of BioMed Central for making this happen.
http://blogs.biomedcentral.com/on-medicine/2016/05/16/timeline-conducting-clinical-trial/

NICE is looking for a Scientific Advisor for a project in collaboration with Myeloma UK, about eliciting patient preferences – note the closing date is 10 June
http://www.jobs.nhs.uk/xi/vacancy/7ca6eff102da72783519befaf5d55a84/?vac_ref=914185982

Both Laura Norburn and Tarang Sharma have alerted us to the following vacancy at NICE, which they are excited about. Be aware the closing date is 10 June.

Scientific Advisor – patient preferences
Science Policy and Research Programme, NICE
£46,625 - £57,640 p.a. (Band 8b)
2 year fixed term, full time
Manchester
Role purpose: to develop and deliver an exploratory study to determine how patient preferences could be captured quantitatively and included in Health Technology Assessment (HTA).

Myeloma UK and NICE are partnering in a 2-year research project to begin exploring how patient preferences can be captured in a more robust quantitative way, in order that data might be incorporated more formally into decision models or be used alongside other evidence as part of decision making process. This is a highly innovative project that seeks to develop methodology that is already used in other disciplines to ensure a fit within the context of existing HTA methods and processes.
This is an exciting opportunity for a researcher with specialist expertise to work with NICE in driving a truly ground-breaking project. The post holder must have extensive knowledge of the methodology relating to the collection and use of patient preferences and be skilled in identifying, critically appraising, analysing and synthesising highly complex evidence, presenting it clearly and precisely both verbally and in writing. An understanding of HTA processes and methodology would be an advantage but is not essential.

The Scientific Advisor will be operationally and technically responsible for developing and delivering the project and seeking funding for further development beyond the initial 2 years. The post holder will also manage the project's advisory body, comprising independent experts and expert organisations, Myeloma UK and other relevant patient groups, NICE staff, and other HTA agencies.

Closing date for applications: 10 June 2016

New SMC guidance to industry on summary information for patient groups
https://www.scottishmedicines.org.uk/Submission_Process/Submission_guidance_and_forms/Templates-Guidance-for-Submission/Templates-Guidance-for-Submission

The SMC Public Involvement team have worked hard with stakeholders to ensure patient groups are provided with better information on new drugs to inform their submissions to SMC. The new 'Guidelines for industry' are online under 'Templates/Guidance Required for a Full submission', although the template remains optional to complete.
Guidance for manufacturers on Summary of Information for Patient Groups
Summary Information for Submitting Patient Groups form

Reflection from EPF about patient involvement in Rapid Relative Effectiveness Assessments

http://www.eu-patient.eu/News/News/time-to-reflect-on-avenues-to-improve-hta-processes2/

This meeting was an opportunity to reflect on the EU Health Technology Assessment (HTA) collaboration, EUnetHTA, which Denmark has coordinated for the last ten years and which is now being taken over by the Netherlands for the joint action 3.
The discussions focused on the opportunities of EU Member States collaborating on Relative Effectiveness Assessments (REAs - assessment of the effectiveness compared with alternative treatments) and the barriers involved

Submitted by Karen Facey, Evidence Based Health Policy Consultant

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Recent publications

Thomas Morel has had the following article published: Quantifying benefit-risk preferences for new medicines in rare disease patients and caregivers. Orphanet J Rare Dis. 2016 May 26;11(1):70
This study (based on a sample of 721 patients and 152 caregivers across 16 UK-based patient associations and 52 rare diseases) aimed to better understand and quantify the preferences and values of rare disease patients and families and to explore to what extent the specific context of disease may alter these preferences and values.
The study supports the hypothesis that patients and their caregivers are willing to accept risks in hopes of finding some benefit, such as a higher chance of drug response or greater health improvement potential. Increasing disease severity, impairment or disability, and the lack of effective therapeutic options were shown to raise significantly the willingness to gain benefit through increased risk.
This study offers an alternative approach to generate robust evidence about patients' preferences and values from a wide range of patients that can ultimately input to complete drug development and approval decision-making processes. One of the conclusions of the study is to suggest a greater role for patient preference studies within the regulatory review process.

http://ojrd.biomedcentral.com/articles/10.1186/s13023-016-0444-9
BMC blog: http://blogs.biomedcentral.com/on-biology/2016/05/27/benefits-risks-preferences-new-medicines-rare-diseases/

Genomics and unlocking the value potential of new technologies was spoken about by Yvonne Bombard in Tokyo during one of the plenary sessions and is the topic of the following articles.

Expectation versus Reality: The Impact of Utility on Emotional Outcomes after Returning Individualized Genetic Research Results in Pediatric Rare Disease Research, a Qualitative Interview Study.
Cacioppo CN, Chandler AE, Towne MC, Beggs AH, Holm IA. PLoS One. 2016 Apr 15;11(4):e0153597. doi:
This is open access.

Understanding the Psychosocial Effects of WES Test Results on Parents of Children with Rare Diseases.
Krabbenborg L, et al. J Genet Couns. 2016 Apr 20. [Epub ahead of print]
The use of whole exome sequencing (WES) for diagnostics of children with rare genetic diseases raises questions about best practices in genetic counselling. While a lot of attention is now given to pre-test counselling procedures for WES, little is known about how parents experience the (positive, negative, or inconclusive) WES results in daily life. To fill this knowledge gap, data were gathered through in-depth interviews with parents of 15 children who underwent WES analysis. WES test results, like results from other genetic tests, evoked relief as well as worries, irrespective of the type of result. Advantages of obtaining a conclusive diagnosis included becoming more accepting towards the situation, being enabled to attune care to the needs of the child, and better coping with feelings of guilt. Disadvantages experienced included a loss of hope for recovery, and a loss by parents of their social network of peers and the effort necessary to re-establish that social network. While parents with conclusive diagnoses were able to re-establish a peer community with the help of social media, parents receiving a possible diagnosis experienced hurdles in seeking peer support, as peers still needed to be identified.

BioMed Central Research Involvement and Engagement

Sophie Staniszewska and Richard Stephens are co-Editors-in-Chief. The journal is "an interdisciplinary, health and social care journal focussing on patient and wider involvement and engagement in research, at all stages. The journal is co-produced by all key stakeholders, including patients, academics, policy makers and service users."
Is this something you would consider being on the Editorial Board of?

Joshua Wamboga, Executive Director of the Uganda Network of AIDS Service Organizations (UNASO) Board Chair Elect, International Alliance of Patients' Organizations (IAPO);A global Patient Voice, would like to share the following with us.

Summary of Key Issues and Demands by patient groups: Uganda's Broken Radiotherapy Machine
Background: For three weeks Uganda's sole radiotherapy machine, housed at Mulago Hospital, has been broken beyond repair. This Cobalt 60 machine was donated to the country in 1995. The machine was already in massive disrepair, with the costly Cobalt Blue (which requires replacing every five years) last replaced in 2002 and constant technical difficulties. 75% of patients with cancer in Uganda need radiotherapy—and this broken machine was providing treatment to more than 100 people with cancer per day. Now that it is broken beyond repair, these patients have no options in Uganda for radiotherapy they need.
In 2013 a replacement machine that uses the same technology--was donated to Uganda, but it requires a new bunker to be built to house the machine—something government has not started to do, and will take at least one year. Importantly, constructing the bunker has been described as a planned output in the Uganda Cancer Institute Budget for years. The more modern machine is a linear accelerator—which does not require a bunker—costing about USD $4.5 million.
This broken machine and the human consequences is just a symbol: of a health system broken beyond repair, and of a government so out of touch with the health needs of its citizens it has failed to develop a plan to prevent catastrophe.
Next steps: Building the bunker will cost about 30 billion UGX according to the Ministry of Health. But money is not the priority issue—it is time. Cancer patients cannot wait the one year for government to finish constructing bunkers that should have been completed years ago.

Recommendations: Parliament must identify a source of funding to send patients to Nairobi for radiotherapy treatment. For example, the budget for VIP health abroad should be ring fenced for this as a matter of urgency, until the bunker has been built. But intervention to address Uganda's multiple health sector emergencies must not stop with the radiotherapy machine. Civil society demands that MPs do not pass the FY2016/17 budget until the following are included:
Primary health care: Enhancement of PHC by 41.2bn in priority, life saving areas in order to support emergency care and treatment and improved administration of health facilities at the local government level.
Health workers: Prioritized enhancement of wages for midwives and other critical cadres of the health workforce at HCs IV and IIIs, estimated to cost Ushs43.179bn. Complete the current recruitment exercise (started in FY 2012-2013) of an additional 3,000 health workers.
Access to medicines: Substantially increase the budget for NMS for essential medicines, in particular doubling the budgeted investment of Ushs 100 billion for HIV and TB medicine—and ensuring those funds are released on time and in full.
HIV treatment stockouts: In FY2015/16 HIV treatment stockouts in public sector clinics caused massive disruptions for people with HIV. Civil society calls on Parliament to urgently pass a supplementary budget to close immediate gaps and to increase the FY 2016/17 budget for HIV treatment accordingly—in order to cater for implementation of the new WHO treatment guidelines indicating all Ugandans should be provided with quality HIV treatment.
Accountability of government: Severe problems are paralyzing the health sector response—in particular ineffective oversight and inefficient management at the executive management level. These problems must be urgently addressed by the Prime Minister and President. Without action, budget absorption and sector performance will continue at the current dismal level.
Access to pay: The limited funding health workers do receive is typically released late, causing demoralization and hardship. Parliament should demand the Ministry of Finance, Planning and Economic Development work with the Ministry of Public Service to ensure health worker salaries are released on time, every month.
Further background: Civil society organizations working to stop maternal, newborn and child mortality and for access to essential health services are urging lawmakers in the waning days of the 9thParliament to pledge

the FY 2016/17 budget addresses the crisis of inadequate motivation, retention, and recruitment of health workers and ensuring an increase in the supply of life saving medicines in public sector clinics.

MPs cannot ignore the health priorities of Ugandans any longer. Without expanded funding for health workers' wages, pregnant women will continue to suffer preventable deaths and complications, people with HIV will wait in line for life saving treatment, and Ugandans will continue to suffer without access to essential health services. For too long, health workers have toiled without adequate remuneration, leading to demotivation, attrition, and lack of accountability for poor quality service delivery. We call on MPs to look at all aspects of the budget for non-priority expenditure that could be re allocated to saving lives by increasing motivation of health workers and supplying clinics with medicines and equipment so that health workers can perform their duties. We demand an increase for PHC funding—the resources health facilities use to pay for electricity, clean water, fuel, and other priorities. The Current PHC non-wage recurrent funding levels are only 41bn instead of the required 82bn—the FY2016/17 budget must correct this.
Government is failing to deliver on its commitment to ensure access to emergency obstetric care in all Health Center IVs, and women are dying as a result. Health facilities cannot respond to the leading causes of preventable maternal death—postpartum hemorrhage, sepsis, obstructed labor, unsafe abortion and eclampsia—unless they are equipped to provide essential health services. Government must match their promises with the funding levels needed to save the lives of pregnant women, newborns and children.

Janet Wale, HTAi PCIG
E-mail: socrates111@bigpond.com