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

Enhanced quality and relevance of HTA through patient and citizen involvement

Welcome to this month's E-Bulletin.

PCIG members may find this new report entitled, '*Principles for Collaborative, Mutually Acceptable Drug Pricing*,' interesting. It concludes the PharmaDiplomacy Dialogue, an EU/US, multi-stakeholder leadership initiative which analyzed how to rebuild trust between the pharmaceutical industry and its key stakeholders, focusing on the critical issue of drug pricing. The report sets out a negotiation framework to navigate the multiple and competing interests of different healthcare stakeholders. It establishes what will be required from payers, pharma and patients to move towards mutually acceptable drug pricing.
http://www.meteos.co.uk/projects/pharmadiplomacy/

Mark Skinner, patient advocate & President, Institute for Policy Advancement, was involved in its development and said at its launch: "Patients have a unique perspective which is distinct from that of pharma, payers and health systems. An inclusive system that values the input of patient perspectives throughout drug development, pricing and its delivery will improve trust. Ensuring alignment on the value of a new therapy requires that patients trust that the value assessment process and their knowing that value was determined based on outcomes directly relative to patients."

Submitted by Karen Facey for Mark Skinner

An example of some of 'the issues' around another huge price jump in the US
EpiPen Furor: Patient Groups Take Money, Stay Mum by Sharon Batt and Adriane Fugh-Berman

The furor around the price of an EpiPen has exposed the contradictions of patient advocacy groups with funding from the pharmaceutical industry. EpiPens contain epinephrine, an oldie-but-goodie, inexpensive generic drug that effectively treats potentially life-threatening allergic reactions. Pharmaceutical companies put this common, inexpensive drug into expensive, auto-injecting devices, which are useful, portable devices for those who are severely allergic to bee stings, peanuts, or other foods.
EpiPen, a Mylan-manufactured auto-injector, has cornered the market, and Mylan has been steadily raising prices. A two-pack of EpiPens, which used to cost about $100, currently runs about $600....
Relatives of people with severe allergies ignited a truly grassroots movement against the price hikes that spread like wildfire over social media, reaching the ears of politicians and creating pressure on the company, which offered to cover more patient copays...
What was really striking was the complete silence about EpiPen prices on the part of the groups trumpeting the importance of allergy awareness, including Food Allergy Research & Education (FARE) (a merger of the Food Allergy & Anaphylaxis Network and the Food Allergy Initiative), the Asthma and Allergy Foundation of America (AAFA) and the Allergy & Asthma Network. All receive substantial funding from Mylan, which lists them as "allies" in its 2015 social responsibility report, and Mylan has given more than $10 million to fund "educational efforts" since 2011....
This is just the latest example of patient advocacy groups failing to speak up when Big Pharma abuses patients' interests. Groups with funding from the pharmaceutical industry rarely, if ever, raise alarms publicly about drug side effects, high drug prices, or inadequate or biased information that the industry provides to consumers – even though these problems are well-documented and can cost lives among members of their constituencies.

Money buys silence. The groups that Mylan funds told *New York Times* reporter Tara Parker-Pope they have had "behind the scenes" conversations with the company to reduce out-of- pocket costs for consumers… Only a handful of patient advocacy and health advocacy groups refuse money from pharmaceutical companies and those groups, including the National Women's Health Network, Breast Cancer Action, and Health Research Group, are the ones critiquing drugs, critiquing biased research, and speaking out about drug prices.

Unfortunately, the public can't always suss out which groups are receiving industry funds because the groups aren't required to declare their corporate funding sources. They should be – although transparency about funding doesn't free a group to speak truth to power. High drug prices help make those generous "educational grants" to patients' groups possible, and may be paying the salaries of their tongue-tied spokespeople.

In the case of the EpiPen, thankfully, angry moms on Facebook are injecting a healthy dose of outrage into the body politic.

Sharon Batt, a social science researcher in pharmaceutical policy, is an adjunct professor in bioethics and a team member with the Technoscience and Regulation Research Unit at Dalhousie University in Canada. Her book on patient group activism will be published in early 2017. Adriane Fugh-Berman is associate professor of pharmacology and physiology at Georgetown University Medical Center and director of Pharmed Out, a Georgetown project that advances evidence-based prescribing and educates health care professionals about pharmaceutical marketing practices.

Published on: August 29, 2016
Published in: Hastings Bioethics Forum

Culture and process change as a priority for patient engagement in medicines development

Marc Boutin, Lode Dewulf, Anton Hoos, Jan Geissler, Veronica Todaro, Roslyn F. Schneider, et al.
Therapeutic Innovation & Regulatory Science August 9, 2016

Patient Focused Medicines Development (PFMD) is a not-for-profit independent multinational coalition of patients, patient stakeholders, and the pharmaceutical industry with interests across diverse disease areas and conditions. PFMD aims to facilitate an integrated approach to medicines development with all stakeholders involved early in the development process. A key strength of the coalition that differentiates it from other groups that involve patients or patient groups is that PFMD has patient organizations as founding members, ensuring that the patient perspective is the starting point when identifying priorities and developing solutions to meet patients' needs. In addition, PFMD has from inception been formed as an equal collaboration among patient groups, patients, and pharmaceutical industry and has adopted a unique trans-Atlantic setup and scope that reflects its global intent. This parity extends to its governance model, which ensures at least equal or greater share of voice for patient group members. PFMD is actively inviting additional members and aims to expand the collaboration to include stakeholders from other sectors. The establishment of PFMD is particularly timely as patient engagement (PE) has become a priority for many health stakeholders and has led to a surge of mostly disconnected activities to deliver this. Given the current plethora of PE initiatives, an essential first step has been to determine, based on a comprehensive mapping, those strategic areas of most need requiring a focused initial effort from the perspective of all stakeholders. PFMD has identified four priority areas that will need to be addressed to facilitate implementation of PE. These are (1) culture and process change, (2) development of a global meta-framework for PE, (3) information exchange, and (4) training. This article discusses these priority themes and ongoing or planned PFMD activities within each.

INTEGRATE-HTA

The European (FP7) INTEGRATE-HTA project developed concepts and methods that enable a patient-centred, comprehensive, and integrated assessment of complex health technologies which were applied in a palliative care case study. Both lay and professional stakeholders were involved throughout the HTA process.

The INTEGRATE-HTA project team has recently produced posters on the key elements/guidances of the project. The posters are now available via our project website. In addition, the project team is organizing a webinar for INAHTA members on 29 September 2016. During this webinar, INTEGRATE-HTA project team members will present the project and the guidances. Two representatives from the Canadian Agency for Drugs and Technologies in Health (CADTH) will share their experiences with using some of the INTEGRATE-

HTA guidances. Finally, there is the opportunity for participants to ask questions to lead researchers of the project. For more information, please visit http://www.inahta.org/2016/07/upcoming-inahta-webinars/

Please keep up to date on our follow-up activities through our project website: www.integrate-hta.eu.

Contributed by Wija Oortwijn, on behalf of the INTEGRATE-HTA project team

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A report from UK, August 2016 that includes key points about HTA in Australia, Canada, Sweden, Germany, France

UK - Breast Cancer Now and Prostate Cancer UK - HTAs
The UK charities Breast Cancer Now and Prostate Cancer UK have published "International Comparisons of Health Technology Assessment," a report that evaluates national HTA systems for funding approval of medicines in regard to patient access to new cancer drugs. The report compares HTAs in England with those in Wales and Scotland and those in Australia, Canada, France, Germany, and Sweden, mainly to explore whether positive features of HTA systems in other countries could be replicated in the UK, particularly to improve patient access to new and innovative cancer drugs. The report claims that, under current systems in the UK, patient access to new innovative cancer drugs is often denied or considerably delayed, and that much can be learned from the systems in other countries. The report's several recommendations include increasing patient involvement, introducing greater flexibility in pricing, and accelerating the process to allow more rapid patient access.
Report @ http://breastcancernow.org/sites/default/files/public/report_final_12_august.compressed_1.pdf

The report is published at a time when the UK's Cancer Drugs Fund has been restructured with a separate budget to improve early access and with the recommendation that all NICE decisions on cancer drugs be published within 90 days of marketing approval. However, the future of the Cancer Drugs Fund remains uncertain, with its fixed budget of GBP340 million potentially limiting its effectiveness in facilitating patient access to innovative drugs.

Contributed by Sally Wortley

In Australia (ISPOR):
Evolution of HTA Guidelines Internationally – Where we are now and where we are heading
ISPOR-Australian Chapter Members are warmly welcome to attend An Afternoon with Mike Drummond. 26 September 2016
Facilitated by Paul Scuffham, members will have the unique opportunity to hear Mike Drummond speak on International HTA Guidelines. There will be a Q&A section with audience members.
http://www.isporac.org/events/mike/

PBAC Guidelines Reviewed – 29 Nov 2016
Lets dissect the newly updated PBAC Guidelines by hearing from the people who matter ….
Tracy Merlin, Andrew Mitchell, Andrew Wilson and Greg Cook along with ISPOR-AC will –

1. *Overview* of the new guidelines (major changes) and *the intent* of the new changes
2. Look at the *key changes* by section
3. Ask what the *evaluators* are looking for?

Join us at Deakin's Burwood Corporate Centre, Melbourne – registrations are open now

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Interesting guidance on patient preferences from FDA

Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and *De Novo* Requests, and Inclusion in Decision Summaries and Device Labeling Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders Document issued on August 24, 2016.
The draft of this document was issued on May 18, 2015. The document will be in effect as of October 23, 2016.

http://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf

Contributed by Karen Facey

Adaptive pathways: key learnings and next steps

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2016/08/news_detail_002586.jsp&mid=WC0b01ac058004d5c1

EMA publishes report on pilot project and will organise workshop in December to further explore concept, 3 August 2016

The European Medicines Agency (EMA) has published a final report on the experience gained during its pilot project on adaptive pathways, a product development concept for medicines that address patients' unmet medical needs.

The pilot project, which has now ended, showed that adaptive pathways can bring multiple stakeholders together – regulators, health technology assessment (HTA) bodies, healthcare professionals and patients – to agree on a prospective plan to generate data on a medicine across its lifespan in areas of unmet medical need. Adaptive pathways can support medicine development in therapeutic areas where evidence generation is challenging, such as infectious diseases, Alzheimer's disease, degenerative diseases, and rare cancers.

Adaptive pathways can be defined as a planned, progressive approach to bringing a medicine to patients. It is not a new route of marketing authorisation; it makes use of existing regulatory tools. Under this approach, the medicine will first be authorised in a small patient population that is likely to benefit most from the medicine. Then, additional evidence is gathered over time resulting in progressive licensing adaptations to extend or restrict the previously authorised indications of the medicine.

Adaptive pathways is still a developing concept which will be refined as more medicines are considered for this approach. The pilot helped to identify a number of aspects for further reflection. These include the need for increased involvement of patients to assist in the selection of candidates for adaptive pathways, the definition of methodologically-sound strategies of real-world evidence collection to support the assessment of both efficacy and effectiveness and the potential involvement of payers - Member States' organisations responsible for decision on pricing and reimbursement – to provide input on pricing strategies.

EMA will further explore adaptive pathways in the context of its parallel advice with HTA bodies, which provides a framework to include additional stakeholders (e.g., patients and, if relevant, payers).Medicine developers who are interested in following the adaptive pathways approach should submit a proposal to EMA. An updated guidance document published today outlines the steps to follow.

To gather the views and proposals from its stakeholders on the adaptive pathways approach, the Agency will organise a workshop on 8 December 2016. Further information on how to register will be published on the EMA website in due course.

It is particularly important that all involved stakeholders agree upfront on a plan of post-licensing knowledge generation for a medicine, before it is authorised, and that the marketing authorisation holder commits to carrying out this plan. Once a marketing authorisation has been granted, the post-authorisation plan becomes a legally binding regulatory obligation. Cooperation between stakeholders and a strong pharmacovigilance system are the basis for a systematic monitoring of the safety and the overall performance of a medicine in clinical practice; these are the two key elements underpinning the adaptive pathways concept.

Bruce Campbell and Paul Knox. Promise and plausibility: Health technology adoption decisions with limited evidence. International Journal of Technology Assessment in Health Care, Published online: Aug 2016

The adoption of new medical devices and diagnostics is often hampered by lack of published evidence which makes conventional health technology assessment (HTA) difficult. We now have 5 years' experience of the Medical Technologies Advisory Committee of the National Institute for Health and Care Excellence (NICE) in the United Kingdom, addressing this problem. This committee assesses devices and diagnostics against claims of advantage, to produce guidance on adoption for the health service.

Results: When scientific and clinical evidence is sparse, promise and plausibility play an increased part in decision-making. Drivers of promise include a clear design and mechanism of action, the possibility of radical improvement in care and/or resource use, and improving health outcomes for large numbers of patients. Plausibility relates to judgements about the whether the promise is likely to be delivered in a "real world"

setting. Promise and plausibility need to be balanced against the amount of evidence available. We examine the influence they may have on decision-making compared with other factors such as risk and cost.
This kind of decision making needs to be transparent and this article explains how these influences can be balanced against the use of more familiar criteria.

What Happens When Underperforming Big Ideas in Research Become Entrenched?

Michael J. Joyner, Nigel Paneth, John P. A. Ioannidis. *JAMA*. Published online July 28, 2016. doi:10.1001/jama.2016.11076
For several decades now the biomedical research community has pursued a narrative positing that a combination of ever-deeper knowledge of subcellular biology, especially genetics, coupled with information technology will lead to transformative improvements in health care and human health. In this Viewpoint, we provide evidence for the extraordinary dominance of this narrative in biomedical funding and journal publications; discuss several prominent themes embedded in the narrative to show that this approach has largely failed; and propose a wholesale re-evaluation of the way forward in biomedical research.

The Journal of Health Organisation and Management has just released a special issue around patient and public participation

http://www.emeraldinsight.com/toc/jhom/30/5

What factors determine the choice of public engagement undertaken by health technology assessment decision-making organizations?

Wortley S, Street J, Lipworth W, Howard K. (2016) Journal of Health Organization and Management 30(6)

Public engagement in health technology assessment (HTA) is increasingly considered crucial for good decision-making. Determining the "right" type of engagement activity is key in achieving the appropriate consideration of public values. Little is known about the factors that determine how HTA organizations decide on their method of public engagement, and there are a number of possible factors that might shape these decisions. This paper seeks to understand the potential drivers of public engagement from an organizational perspective.
Design/methodology/approach
The published HTA literature is reviewed alongside existing frameworks of public engagement in order to elucidate key factors influencing the choice of public engagement process undertaken by HTA organizations. A conceptual framework is then developed to illustrate the factors identified from the literature that appear to influence public engagement choice.
Findings
Determining the type of public engagement to undertaken in HTA is based on multiple factors, some of which are not always explicitly acknowledged. These factors included the: perceived complexity of the policymaking issue, perceived impact of the decision, transparency and opportunities for public involvement in governance, as well as time and resource constraints. The influences of these factors vary depending on the context, indicating that a one size fits all approach to public engagement may not be effective.
Originality/value
Awareness of the various factors that might influence the type of public engagement undertaken would enable decision-makers to reflect on their choices and be more accountable and transparent about their choice of engagement process in eliciting public values and preferences in a HTA organization.

Bulletin du Labo du partenariat / Partnership Lab Bulletin
Août/August 2016
Le **Labo du partenariat** (dirigé par Dr. Antoine Boivin) produit une veille électronique mensuelle qui vise à faciliter l'accès aux nouvelles publications sur l'engagement des patients et du public. Si vous ne souhaitez pas recevoir le bulletin, n'hésitez pas à nous écrire.

The **Partnership Lab** (lead by Dr. Antoine Boivin) is producing a monthly electronic newsletter that seeks to facilitate access to new publications on public and patient engagement. If you do not wish to receive the newsletter, please send us an email.

Engagement en recherche / Engagement in research

How and why should we engage parents as co-researchers in health research? A scoping review of current practices (Libre accès/Open access)
Shen et al. (2016) *Health Expectations*

Supporting public involvement in interview and other panels: A systematic review (Libre accès/Open access)
Baxter et al. (2016) *Health Expectations*

Power to the people: To what extent has public involvement in applied health research achieved this? (Libre accès/Open access)
Green (2016) *Research Involvement and Engagement*

Lay involvement in the analysis of qualitative data in health services research: A descriptive study (Libre accès/Open access)
Garfield et al. (2016) *Research Involvement and Engagement*

Models and impact of patient and public involvement in studies carried out by the Medical Research Council Clinical Trials Unit at University College London: findings from ten case studies (Libre accès/Open access)
South et al. (2016) *Trials*

Engaging patients and consumers in research evidence: Applying the conceptual model of patient and family engagement (Libre accès/Open access)
Carman & Workman (2016) *Patient Education and Counseling*

Sharing experiences and expertise: The health care systems research network workshop on patient engagement in research (Libre accès/Open access)
Madrid et al. (2016) *Journal of Patient-Centered Research and Reviews*

Public engagement and deliberation in human embryo research governance in Australia 2001–2011
Dodds & Ankeny (2016) *Big Picture Bioethics: Developing Democratic Policy in Contested Domains*

The science and art of partnering with patients in research
Donnelly & Brady (2016) *Shared Decision Making in Health Care*

Culture and process change as a priority for patient engagement in medicines development
Boutin et al. (2016) *Therapeutic Innovation & Regulatory Science*

What factors determine the choice of public engagement undertaken by health technology assessment decision-making organizations?
Wortley et al. (2016) *Journal of Health Organization and Management*

Stakeholder involvement in health technology assessment at national level: A study from Iran
Yazdizadeh et al. (2016) *International Journal of Technology Assessment in Health Care*

Adapting conceptual frameworks for patient engagement in emergency department research
Wright et al. (2016) *Academic Emergency Medicine*

Developing a model to enhance patient involvement in designing and conducting research: Views of patients, academics and health service staff
Evans et al. (2016) *Emergency Medicine Journal*

Engagement dans la transformation des systèmes de santé / Engagement in health-system transformation

User involvement in the implementation of clinical guidelines for common mental health disorders: A review and compilation of strategies and resources (Libre accès/Open access)
Moreno & Moriana (2016) *Health Research Policy and Systems*

Service user involvement for mental health system strengthening in India: A qualitative study (Libre accès/Open access)
Samudre et al. (2016) *BMC Psychiatry*

Participation and trust: Conditions and constraints on democratic deliberation
Dodds (2016) *Big Picture Bioethics: Developing Democratic Policy in Contested Domains*

Designing services in partnership with patients
Goodrich (2016) *Nursing Times*

Engagement en santé des communauté / Engagement in community health
Strategies to empower communities to reduce health disparities
Thompson et al. (2016) *Health Affairs*

Power and glory: Applying participatory action research in public health
Baum (2016) *Gaceta Sanitaria*

Engagement en enseignement / Engagement in teaching
Patients and families as teachers: A mixed methods assessment of a collaborative learning model for medical error disclosure and prevention
Langer et al. (2016) *BMJ Quality & Safety*

A collaborative turn: Trends and directions in community engagement
Saltmarsh (2016) *Learning Through Community Engagement*

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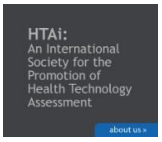

HTAi 2017 Rome, June 17-21, 2017
http://www.htai.org/meetings/annual-meetings/htai-2017-rome.html

Abstract Submissions
Workshops & Panels, December 2, 2016
Posters & Oral Presentations, January 6, 2017
Travel Grants: Application Deadline, January 6, 2017
Registration: Early Bird Deadline, March 3, 2017

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