NEWS

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

HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) E-Bulletin, November 2017

Enhanced quality and relevance of HTA through patient and citizen involvement

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

Brazil, October '17

- HTAi2018 Annual Meeting update
- What's happening – in the world of patient and public involvement
- Patient Focused Medicines Development - Public Consultation on Patient Engagement Quality Guidance
- News
- Publications

Welcome to this month's E-Bulletin

Strengthening the Evidence-to-Action Connection: HTAi2018 Annual Meeting update

Registration will open at the beginning of January 2018.
We expect around 1000 researchers, policy makers, payers, industry, academia, health service providers, agencies, and patients/consumers at our Annual Meeting in Vancouver, BC, Canada on June 1-5, 2018 to discuss ways to inform policy, healthcare decisions and the role of health technology assessment (HTA).
HTAi2018.org #HTAiVancouver2018

Now closed: calls for abstracts for contributions to the Scientific Programme and HTAi Travel Grant applications. Please keep us informed about successful applications. This helps us to plan for the Annual Meeting.

Can we make this a 'Patients Included' conference?

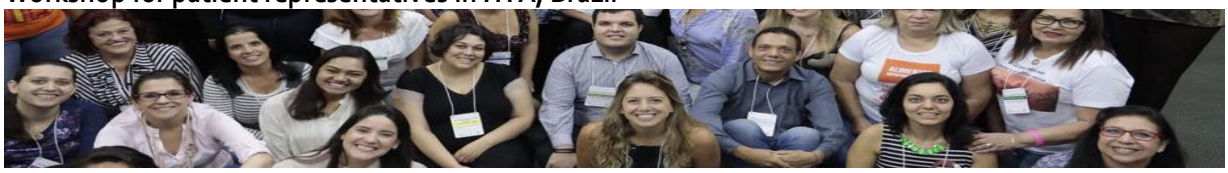
For this it needs to be able to demonstrate that the event is committed to incorporating the experience of patients as experts in living with their condition while ensuring they are neither excluded nor exploited. Self-assessment must meet five clauses, that: Patients or caregivers with experience relevant to the conference's central theme actively participate in the design and planning of the event; participate in its delivery, and appear in the physical audience. Disability requirements of participants are accommodated, and all applicable sessions are open to patient delegates. Travel and accommodation expenses for patients or carers participating in the advertised programme are paid in full, and scholarships are provided for delegates. Free streaming video is provided online wherever possible.

https://patientsincluded.org/conferences/

2018 HTAi Global Policy Forum background paper
Thank you to those who commented on the draft background paper, 'Facing the dynamics of future innovations: The role of HTA, industry and health system in scanning the horizon', has been prepared to inform discussion during the HTAi Global Policy Forum. The meeting will take place from January 28 to 30, 2018, in Barcelona, Spain for members of the Policy Forum.

What's happening – in the world of patient and public involvement

Workshop for patient representatives in HTA, Brazil



In October 2017, the Executive Secretariat of the National Committee for Health Technology Incorporation (CONITEC) held a workshop intended for patient representatives as a plan to improve patient and public involvement in HTA process. There were 91 participants representing several diseases and regions of Brazil. This action allowed knowing the patient organizations. Moreover, through various participatory activities, it was possible to disclose information about HTA and CONITEC's actions to the public, who made suggestions to improve patient and public engagement in the process of Technology Incorporation in Brazil."

Contributed by Aline Silveira Silva, Brazilian Ministry of Health
http://conitec.gov.br/ultimas-noticias-3/16795-forum-discute-participacao-social-na-incorporacao-de-tecnologias-em-saude

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Rapid qualitative evidence synthesis in HTA – the Canadian Agency for Drugs and Technologies in Health (CADTH)
Last month, Andrea Smith (CADTH) presented interim results of a collaborative project exploring published guidance and examples of rapid qualitative evidence synthesis at the 23rd Qualitative Health Research Conference in Quebec City, Canada. Her presentation described an ongoing scoping review being conducted collaboratively by CADTH (Laura Weeks, Andrea Smith, David Kaunelis) and the University of Sheffield (Andrew Booth and Fiona Campbell). Increasing requests to include qualitative evidence as part of health technology assessment have given rise to a rapid qualitative evidence synthesis as an emerging field of methodological development, yet little guidance exists. Through this review we aim to identify the extent to which current guidance and practice offers a methodological evidence base to facilitate common expectations and methods for a rapid qualitative evidence synthesis process.

Contributed by Laura Weeks, CADTH in Canada

Seeking Your Input: Developing an evaluation guide to help strengthen patient groups' submissions
Please provide your comments via email to pcodrinfo@cadth.ca by **5 pm Eastern Time on December 4, 2017. Please note that a response to these questions will serve as implied consent to participate in this quality improvement initiative.**

CADTH and the Canadian Cancer Action Network (CCAN) have fostered initiatives to improve the dissemination of patients' experiences and make CADTH's pan-Canadian Oncology Drug Review (pCODR) process more comprehensive and transparent. As part of this work, a dynamic framework to assess the longitudinal impact of the patient evidence on the HTA process submitted to the pCODR program is being

developed in collaboration with the Canadian Centre for Applied Research in Cancer Control (ARCC). The objectives of this collaboration include:

- Providing assistance to patient groups with navigating vidence submission process for drug reviews
- Enhancing the quality of evidence submissions
- Fostering enhanced engagement of patient groups in the pCODR process

The framework is intended to ensure that meaningful outcomes are achieved to advance patient input and perspectives in the cancer drug review processes. In an effort to establish a holistic evaluation of how patient evidence submissions help to inform cancer drug funding recommendations, the framework consists of three main elements for assessment:

- Impact on patient groups' knowledge and understanding of the evidence submission process
- Impact on the quality of patient evidence submissions
- Impact on the quality of patient experience in the pCODR process

One of the initiatives under this framework is to develop and provide patient groups with a submission guide to aid in the synthesis of patient and caregiver experiences that are used to inform the pCODR Expert Review Committee's (pERC) recommendation on cancer drugs. The values elicited from the patient submissions are used by pERC to understand patient and caregiver experiences and perspectives on their illness and treatments.

Requesting Input from Patient Groups

We are seeking your opinion regarding a draft of the submission guide, which is being developed by the pCODR program in collaboration with ARCC and CCAN. You will be asked to answer two questions to determine how the proposed guide could best support the patient submission. Please use the following template to respond to the key questions outlined below.

Questions for consideration:

1. When preparing your patient submission, are there key areas that you feel take a greater deal of effort to undertake. Please choose all that apply. Please provide details with regards to the challenges that you experienced.

a) Developing a survey to gather patient data
b) Developing a survey to gather caregiver data
c) Use of patient/caregiver quotes
d) Analysis of data
e) Presenting analysis and themes
f) Selecting the most important information that reflects data collected
g) Other; please specify

2. As a key objective of the proposed guide is to assess the impact of the patient evidence submission that is used to inform pERC's recommendation on cancer drugs, what information would be of most interest and valuable to you to have in the guide (for example, guidance on the balance of quantitative/statistical information such as frequencies versus qualitative testimonials)? Please explain and provide examples.

Background: How Patient Input is used in the pCODR Process

We ask for input early in the review process as this helps to develop the review protocol and ensure that issues that are important to patients and caregivers are considered in our review. With regards to the pCODR process, feedback is used by pERC to further consider values that are important to patients and caregivers, and help to improve clarity of the recommendation. This information is also shared with the participating public drug programs of the ministries of health and cancer agencies.

Submitted by Melissa Sullivan and Deb Maskens

The Added Value of Patient Organisations

http://www.eu-patient.eu/globalassets/library/publications/epf_added_value_report_final.pdf

The report is informative and includes the following discussion.

The roles that patient organisations play are constantly changing and evolving. Currently, patient organisations are very much demonstrating their value as co-designers, facilitators, conveners, innovators, policymakers, peer supporters, researchers, communicators, data-collectors as well as service providers and advocates. They can be seen as the glue that binds public and private activity together. Yet when dealing with other institutions, there is often a systematic failure of cooperation and a tradition of tokenism in working with patient organisations.
The value of patient organisations is in working within the core principles of representing, mobilising and empowering patients and advocating their rights.
The four major areas where patient organisations have unique value are: (1) policy (helping policymakers understand patient priorities and experiences of living with a disease/condition; and as end-users in health services); (2) capacity-building and education (strengthening organisational management and governance, producing and reviewing health-related information, improving health literacy and knowledge about patients' rights); (3) peer support (and networking); and (4) research & development (as active research collaborators, in regulatory process, and through data collection).

Challenges for patient organisations relate to limited resources, both human and financial, lack of organisational professionalism and lack of performance measurement. External challenges include lack of legally-acknowledged right to public funding, systemic failure of collaboration, tokenism and legislative gaps.

- Patient organisations are subject to impact measurement, which is unfair. This level of scrutiny is not routinely applied to other health stakeholders such as the pharmaceutical industry, health insurers or health professionals' associations. The concept of things being done to patients, and not with still exists.
- Yet most popular impact assessment tools do not measure the weight of the contribution of a single stakeholder in a final outcome; neither do they consider measuring the concrete effect of meaningful involvement of patients or civil society representatives as such.
- Patient organisations often lack the mechanisms to develop or strengthen their strategic planning, improve their organisational development and capacity for fundraising, and professionalise their organisational behaviour. Reasons include a lack of earmarked funding, understaffing, a lack of particular skills, or an underdeveloped knowledge base (where the patient organisation does not have access to information on how to strengthen and professionalise its organisation).
- The technical and bureaucratic language used is a barrier, as is a relatively strong negative institutional culture and tradition of tokenism when it comes to involvement of patient organisations.
- The lack of explicitly earmarked budgets devoted to systematic involvement of individual patients and patient groups is a barrier. Until recently, most health budgets focused on addressing diseases: deliberating who to involve and how in tackling their determinants, management and treatment is a relatively new exercise in health policy-making. Members of patient organisations are patient representatives, often operating on a voluntary basis, are often seen as not expert
- Another barrier is the lack of EU legislation on meaningful patient involvement and systematic cooperation in contexts beyond specific clinical or medical issues (this includes references to patients in legislation governing health or research budgets and priorities, transparency and governance issues etc).

The question is how to gain greater professionalization without losing patient representativeness; decreasing reliance on grassroots and increasing dependence on experts as well as elite-level contacts. We need to retain the unique value of patient organisations.

Groups put the majority of their time and resources into activities aimed to influence others and not into membership-focused activities.
Until recently, most health budgets focused on addressing diseases. Deliberating on who to involve and how in tackling their determinants, management and treatment is a relatively new exercise in health policy-making. Patient representatives, often operating on a voluntary basis, are often seen not as expert figures

and subsequently are denied the right to financial compensation for their time and contribution to various activities.
Innovative and ethical options for funding patient groups - that will enable them to function and maintain their independence from industry and from government - are needed for them to be credible stakeholders and partners in health policy debate.

Authors: Dorota Sienkiewicz, Corine van Lingen. Editors: Nicola Bedlington, Camille Bullot, Kaisa Immonen. The EPF 2017 Work Programme received funding from the European Union in the framework of the Health Programme.

Where are our quality measures for PPI done well (to be completed by all parties)?
Two closely related lectures, published online as blogs by a Canadian patient advocate. One is in service delivery and the second in research.
http://johannesen.ca/2017/10/two-way-street-critical-look-patient-family-engagement/
http://johannesen.ca/2017/11/exploring-purpose-meaning-patient-engagement-pediatric-neurodisability-research/
Jennifer states that: We have been working at this project of 'patient engagement' for a decade or more. If healthcare really cared, we would be much further along by now. Why are we still talking about what 'meaningful' looks like? Why are patient experts still not paid for their expertise? Why have we not developed a body of research to quantify the value of patient engagement?
'Patient engagement' was originally adversarial (the AIDs movement), as is often required for disruptive social movements, but has become a healthcare initiative, as if they invented it. Our presence achieves something for the institution that has nothing to do with what we accomplish.
As 'patient engagement' participants in health services, hand-selected by the institution, assigned to activities they've pre-approved, we function as enablers, supporters, and cheerleaders. Institutions need to meet regulatory requirements or achieve accreditation, or a funder's criteria, or satisfy public demand.
You could say that: The purpose of patient engagement in research is to extract symbolic value from patients in order to access funding and enhance legitimacy of research projects. Positive outcomes and good feelings are incidental. Researchers need to meet funders' or publishers' criteria. There is moral value in patient engagement, 'we care about our patients' – because it's principles-based, there has been no need to substantiate it via outcomes.

'How do we do it better', and 'why do we do it at all'? What is the purpose and the meaning of patient engagement?
A literature review* related to patient engagement in research found virtually no evidence, but lots of hypothesized (or imagined) impacts: better quality research (more relevant and responsive to patient needs); expanded applicability (hard-to-reach populations); empowering patients; increased translation, dissemination and update of results; democracy and accountability; fulfilling a moral obligation. Virtually none of this has been validated - it's not measured
Why do patients want to be involved - patient engagement helps patients feel they have a voice - only the ones who are involved, that is. Patients are grateful. We are also flattered – often we're invited to join these projects personally, and it feels good to be recognized as someone whose ideas are valued. We are also granted a special elevated status – participating behind the scenes potentially gives us access to information that the general patient population doesn't get. We get a sense of community.
We are also keen to make a difference. And we know that so much of healthcare relies on volunteers to get important things done; some families experience things they hope others will never have to endure – so they may participate to spark change.
These motivations of ours are often borne out of deeply personal and perhaps traumatic experiences. We make an ideal group of people who can be relied upon to give generously and continuously; and we are vulnerable. Terms of patient engagement are purely relational – and there are no rules.

'Patient engagement' is a leaderless movement and there's no one pulling the strings. Here's how we know that patient engagement in research is largely symbolic and not intended to be consequential: lack of literature; no quality or protocol oversight- no requirement for quality; no protocols to govern how patients in advisory roles are selected and trained; no mandate for patient advisors to be representative of patient populations; no direction on how their perspectives are elicited and integrated; no measurement; no compensation.
Only the researcher or staff member is paid for the work and gets reputational credit in ways that are relevant to them – literature, protocols, measurement, funding – none of it applies to patient engagement. It surprises me deeply that there is no oversight governing the involvement of patients and families in advisory roles.
Ways forward for researchers and others: professionalize and train patients; stop privileging certain patient voices over others because it's easier, and stop trading on personal relationships; appropriate paid training for patients - it's up to you to decide patient advisory is worth the investment; get clear on patient representation - who and what advisors represent - and be transparent about it; be accountable, make sure that patients understand what they are there for and what you want from them; produce a rationale and evidence to justify any patient engagement; contribute to the literature on patient engagement

If institutions were interested, they would hire patients in management positions. They would pay us appropriately for our expertise. They would integrate patient advisories at every level of the institution, and patients would be deeply embedded in all processes.

*L Esmail, E Moore, A Rein. Evaluating patient and stakeholder engagement in research: moving from theory to practice. J Comp Eff Res. 2015 Mar;4(2):133-45. doi: 10.2217/cer.14.79.

Contributed by Anna Scott

Patient Focused Medicines Development - Public Consultation on Patient Engagement Quality Guidance
Open until 20.12.2017
Unlocking potential shared value with The Synergist: http://patientfocusedmedicine.org/public-consultation/

What's happening – News

Amsterdam named as the new home of the European Medicines Agency (EMA)
The Agency will move its headquarters from the current base in London, UK, by the March 2019, as a direct consequence of the UK's withdrawal from the European Union. The EMA has been based in London's Canary Wharf since it was established in 1995.

New 'Physician's Pledge'
This is a revised version of the one adopted in the World Medical Association Declaration of Geneva 1948
Sources: https://www.wma.net/news-post/modern-physicians-pledge-approved-by-world-medical-association/ (comment) and https://www.wma.net/policies-post/wma-declaration-of-geneva/ (declaration)

The three big new items with high relevance for the patients are:

- The health and well-being of my patient will be my first consideration;
- I will respect the autonomy and dignity of my patient;
- I will attend to my own health, well-being, and abilities in order to provide care of the highest standard.

Submitted by Anke-Peggy Holtorf

What's happening – Publications

Rheumatoid arthritis patients treated in trial and real world settings: comparison of randomized trials with registries.
Rheumatoid arthritis patients treated in trials and real world settings differ
Rheumatology 2017. Nov 14. doi: 10.1093/rheumatology/kex394. [Epub ahead of print]
Kilcher G, Hummel N, Didden EM, Egger M, Reichenbach S; GetReal Work Package 4.
There are substantial systematic differences in patient characteristics between RCTs and registries in RA. The efficacy seen in RCTs may not reflect real-world effectiveness.

Evaluating patient and public involvement in health research: from theoretical model to practical workshop. A Gibson, J Welsman, N Britten. Health Expectations 2017:20(5):826–835.
At the core of successful PPI is the dynamic interaction of different forms of knowledge, notably lay and professional. We have developed a four-dimensional theoretical framework for understanding these interactions.

Is it worth it? Patient and public views on the impact of their involvement in health research and its assessment: a UK-based qualitative interview study
JC Crocker, A-M Boylan, J Bostock, L Locock. Health Expectations 2017;20(3):519–528
Explores the views of PPI contributors involved in health research regarding the impact of PPI on research, whether and how it should be assessed.

Measuring what matters to rare disease patients – reflections on the work by the IRDiRC taskforce on patient-centered outcome measures
T Morel and SJ Cano Orphanet Journal of Rare Diseases (2017) 12:171
DOI 10.1186/s13023-017-0718-x
Our ability to evaluate outcomes which genuinely reflect patients' unmet needs, hopes and concerns is of pivotal importance. However, much current clinical research and practice falls short of this objective by selecting outcome measures which do not capture patient value to the fullest. In this Opinion, we discuss Patient-Centered Outcomes Measures (PCOMs), which have the potential to systematically incorporate patient perspectives to measure those outcomes that matter most to patients.

URL to the article is: https://ojrd.biomedcentral.com/articles/10.1186/s13023-017-0718-x or http://rdcu.be/x3pz

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Disentangling patient and public involvement in healthcare decisions: why the difference matters
M Fredriksson and JQ Tritter. Sociology of Health & Illness 2017; 39(1):95–111.
We analyse crucial distinctions between patient involvement and public involvement using examples from Sweden and England. We highlight that patients have sectional interests as health service users in contrast to citizens who engage as a public policy agent reflecting societal interests. Patients draw on experiential knowledge and focus on output legitimacy and performance accountability, aim at typical representativeness, and a direct responsiveness to individual needs and preferences. In contrast, the public contributes with collective perspectives generated from diversity, centres on input legitimacy achieved through statistical representativeness, democratic accountability and indirect responsiveness to general citizen preferences. Thus, using patients as proxies for the public fails to achieve intended goals and benefits of involvement.

Related: M Fredriksson, M Eriksson, JQ Tritter. Scandinavian Journal of Public Health2017. Involvement that makes an impact on healthcare: Perceptions of the Swedish public. **First Published November 1,**

2017 . More Swedish people have confidence in impact of participating as individual patients rather than collectively and as citizens.

Submitted by Russell McGowan

Ethical principles for the use of human cellular biotechnologies
PR Wolpe, KS Rommelfanger and the Drafting and Reviewing Delegates of the BEINGS Working Groups.
Recent developments in bioengineering promise the possibility of new diagnostic and treatment strategies, novel industrial processes, and innovative approaches to thorny problems in fields such as nutrition, agriculture, and biomanufacturing. As modern genetics has matured and developed technologies of increasing power, debates over risk assessments and proper applications of the technology, and over who should have decision-making power over such issues, have become more prominent. Recently, some scientists have advocated that ethicists "step out of the way," whereas others have called for greater ethical scrutiny, or even for moratoria on some lines of research1,2. As a community, however, we must together determine the proper application of these powerful biological tools. This paper, a consensus statement of a group of interdisciplinary delegates drawn from the top biotech-producing countries of the world, offers a set of ethical principles to contribute to the ethical conversation about human cellular biotechnological research moving forward.

Open access .pdf at https://www.nature.com/articles/nbt.4007.pdf

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