NEWS

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

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

The Steering Committee is about to have its first teleconference of the year where it will address expanding the committee and its application for 2018 Interest Group funding, a small amount of funding made available to all Interest Groups.

The PCIG is also part of the Innovative Medicines Initiative (IMI) Patients Active In Research and Dialogues for an Improved Generation of Medicines (PARADIGM), March 2018-September 2020:

- prioritisation of research
- **early dialogue between regulators and HTA**
- design of clinical trials

The project will develop consensus-based recommendations on processes, tools, templates and methods to measure and demonstrate the added value of innovative and effective approaches to patient engagement.

The IMI is a public-private partnership of the European Commission and the European Federation of Pharmaceutical Industries and Associations (EFPIA). www.imi.europa.eu/

The HTAi Secretariat is looking for HTAi membership when making changes to the listserv, so please be aware of this: https://www.htai.org/membership/2018-and-20182019-membership/

HTAi 2018 Annual Meeting
https://www.htai2018.org/#
Join HTAi 2018 to engage with an extensive network of leaders, experts and key policymakers in lively discussions around the theme of the meeting.
HTAi welcomes those invested in the production and implementation of HTA from Canada, neighbouring countries and around the world.

PCIG Workshop: PRE-ANNUAL MEETING WORKSHOPS DAY 2: SATURDAY, JUNE 2
Full Day session from 08:30 to 16:30
WS13: Building a Shared Resource of Patient Experience and Preferences to Improve HTA
This workshop presents the issue of the burden of patient input in HTA and seeks to identify solutions by developing participants knowledge of common gaps, issues and overlaps in patient input sought for HTAs,

awareness of existing shared resources of patient experience and how they function, ability to identify potential collaborators and approaches to building shared resources such as repositories. This workshop explores opportunities to reduce the burden of patient input in HTA for patients and patient groups, and increase its value through creating shared resources. Participants will gain awareness of existing resources for capturing and sharing patient experience and preferences, and approaches to tackling the burden of patient input; an understanding of the requirements for capturing and sharing patient experience for HTA; and experience of collaborating to scope potential solutions to the issue.
Early Bird Registration ends on March 31, 2018
All prices are displayed in US dollars. Social Event Costs Include 5% GST
Regular Registration until May 18, 2018, Midnight EST

In the next couple of months we will be highlighting HTAi 2018 panel sessions, meetings and oral presentations that PCIG members are involved in – so please send in information so that we can include it.

What's Happening:

Legislative proposal for EU collaboration on HTA published
https://ec.europa.eu/health/sites/health/files/technology_assessment/docs/com2018_51_en.pdf

Read the European Patient Forum's position on the new EC proposed legislation for European collaboration in HTA where the envisioned involvement of patients is welcomed.
http://www.eu-patient.eu/News/News/future-european-cooperation-on-hta-a-step-forward-in-improving-healthcare-for-all/

Valentina Strammiello, European Patients Forum (EPF)

The draft had been leaked by @POLITICOEurope. The European Public Health Alliance has an informative review and opinion on the draft at https://epha.org/the-top-5-issues-in-medicines-policy-for-2018/?utm_source=POLITICO.EU&utm_campaign=846defd51d-EMAIL_CAMPAIGN_2018_01_24&utm_medium=email&utm_term=0_10959edeb5-846defd51d-189813045

Patient and Caregiver Preference Study

The Amyloidosis Research Consortium UK is undertaking a patient and caregiver preference elicitation study in hereditary ATTR amyloidosis (hATTR). The study seeks to better understand how hATTR patients and caregivers perceive the potential benefits, risks and disadvantages associated with the types of characteristics of new treatments, and to explore the trade-offs involved. ARC UK intends to use this data to inform upcoming HTA evaluations of two new treatments for this ultra-orphan disease. ARC UK is currently seeking a collaborating research organisation / expert to conduct the study. If you are interested or for more information please get in touch with ARC UK by emailing Sarah Richard at srichard@arci.org.uk

Sarah Richard

Taking Action on Access - Drug Iceberg 2.0 Report Published
IPPOSI is pleased to announce the publication of the "Drug Iceberg" 2.0 report. Delivered in partnership with the Medical Research Charities Group (MRCG), the report is a February 2018 update on the well-received first 'Drug Iceberg' report. The 2.0 report contains:

- Key recommendations - including a call for the development and implementation of a new national strategy on access to medicines in Ireland.
- A list of relevant developments that have occurred since the publication of the first 'Drug Iceberg' report (August 2017).

- The summarised discussion points from a multi-stakeholder Round Table meeting in October 2017.
- Four international case studies examining Access to Medicines in other jurisdictions.
- A list of achievable target outcomes for 2018 as part of IPPOSI and MRCG actions.

The report is the first output from IPPOSI's 2018 Theme of 'Taking Action on Access'

Dr John FP Bridges will take up a new position as Professor in the Departments of Biomedical Informatics and Surgery within the Ohio State University College of Medicine.

At OSU he will be core faculty within the newly created Center for the Advancement of Team Science, Analytics and Systems Thinking in Health Systems Research and Implementation Science (CATALYST) and the Center for Surgical Health Assessment, Research and Policy (SHARP). At OSU John will focus on conducting research to promote the translation and implementation of advances in medicine through informed decision making. He will continue to conduct research to advance and apply methods to promote a greater understanding of the preferences of patients and other stakeholders in medicine, especially as it relates to regulatory science. He will also build new partnerships to promote precision medicine, including demonstrating how "economics" can contribute to a multi-omics view of human disease and treatment. If you need to find John his new email is bridges.167@jhu.edu

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AllTrials: Time is up for clinical trials that fail to publish results

For several years AllTrials has been asking the FDA to censure sponsors who break the law in failing to report clinical trial results. The FDA has assured us, publicly and privately, that new clarifying rules - the FDAAA Final Rule - will help them do so but though there are trials on ClinicalTrials.gov whose results are years overdue, to date they have not issued a single fine, despite the power to levy up to $10,000 per day.

So we have sent an open letter to the FDA telling them that today is the launch of an an online tracker (FDAAA.trialstracker.net) so that we can all see who is late according to the FDA's own registry data. Today is important - it is 13 months since the FDAAA Final Rule that made it crystal clear who needs to report results within a year of a trial ending (13 months includes the extra 30 days grace FDA allows for administration issues). We thought you would like early sight of this letter as the tracker launches: http://www.alltrials.net/news/open-letter-to-the-fda

We are going to write to them every week from now on with a list of those trials that breach the reporting requirements, as well as a rolling total of the fines they could be levying. We will succeed in recovering missing trials. Some AllTrials supporters have already started a fund to help in achieving this. If you can make any contribution towards its target, that would be wonderful.

Ben & Síle (UK)

EUPATI

EUPATI Toolbox now also includes a minicourse (or starter kit) about community advisory boards or CABs. CABs are patient community-led, neutral spaces where stakeholders in medical research and development, and clinical trials can come together and discuss scientific (or even policy) matters and questions.
EUPATI is a public-private partnership operated and led by the European Patients' Forum

EUPATI webinar on experiences with patient involvement in HTA processes. We don't stop there, and there will be much more coming on registries and other key aspects of patient involvement in research and clinical trials. https://vimeo.com/251519710

April 18 Bruxelles (Belgium): European Patients' Rights Day 2018, Join Us!

Active Citizenship Network (ACN) of Italy will celebrate the 12th European Patients' Rights Day (EPRD) with

a multistakeholder conference to be held on 18th April 2018 in Bruxelles.
The aim of the convention is to debate, inform and exchange good practices on therapeutic adherence, sharing and discussing the different experiences and more effective solutions in overcoming some of the existing barriers to appropriate prescription and adherence to pharmacological medical plans. **Read More** CLICK HERE to participate

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FDA on patient engagement

Patient-Focused Drug Development – Collecting comprehensive and representative input, December 18, 2017
https://www.fda.gov/downloads/Drugs/NewsEvents/UCM589699.pdf

Publications

Anke-Peggy Holtorf 1, Nigel Cook 2. Chapter 18. The Role of Patients in Market Access

1 Health Outcomes Strategies GmbH, CH 4055 Basel
2 Head of Decision Support & Insights, Global Patient Access, Novartis Pharma AG, CH4056 Basel
Our chapter has just been published in the book:
Kockaya G, Wertheimer A. Pharmaceutical Market Access in Developed Markets. SEEd: Torino, 2018
It is open access and can be downloaded at this link:
http://books.seedmedicalpublishers.com/index.php/seed/catalog/book/Pharmaceutical_MA_developed

Nigel Cook

Patient-Centered Outcome Measures

Thomas Morel and Stefan J Cano. Measuring what matters to rare disease patients – reflections on the work by the IRDiRC taskforce on patient-centered outcome measures. Orphanet Journal of Rare Diseases (2017) 12:171
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. We argue for greater multi-stakeholder collaboration to develop PCOMs, with rare disease patients and families at the center. Beyond advancing the science of patient input, PCOMs are powerful tools to translate care or observed treatment benefit into an 'interpretable' measure of patient benefit, and thereby help demonstrate clinical effectiveness. We propose mixed methods psychometric research as the best route to deliver fit-for-purpose PCOMs in rare diseases, as this methodology brings together qualitative and quantitative research methods in tandem with the explicit aim to efficiently utilise data from small samples. And, whether one opts to develop a brand-new PCOM or to select or adapt an existing outcome measure for use in a rare disease, the anchors remain the same: patients, their daily experience of the rare disease, their preferences, core concepts and values. Ultimately, existing value frameworks, registries, and outcomes-based contracts largely fall short of consistently measuring the full range of outcomes that matter to patients.

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