

HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) E-Bulletin, January 2020

Enhanced quality and relevance of HTA through patient and citizen involvement

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

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Welcome to our E-Bulletin and the Year of the Rat!

From our Chair

Late in January, the HTAi Patient and Citizen Involvement Interest Group (PCIG) held its first Steering Committee meeting for 2020. The focus of the meeting was projects. While we identified further improvements to the update process in our new structure, it was great to hear what the projects have been achieving. We are putting in place a process to ensure we share information from projects in this bulletin, but in the meantime I'm pleased to share the following.

-Work developing a template to provide patients and patient groups with summary information about products being assessed by HTA bodies continues apace to the point where we're looking at how we store and share all the outputs including surveys

- ✚ Work to describe patient participation at the organizational level is now at the stage of testing the questionnaire that will be used for targeted interviews with HTA bodies and patient representatives
- ✚ Different stakeholders' stories of impact are being collected to aid reflection on patient involvement in HTA, while the project sub-committee works to recruit a social scientist and patient representative
- ✚ Analytical frameworks are being reviewed for work to conduct a landscape analysis of routes to patient involvement in low and middle income countries
- ✚ Sample documents collected from HTA bodies are informing the drafting of the tools to support patient engagement in Early Dialogues/Scientific Advice as part of the PARADIGM project
- ✚ The Patient Preferences Project Sub-Committee is developing webinars (details shortly) to raise awareness and share knowledge from the wide variety of initiatives in this area
- ✚ Finally, the Gaps and Resources Project Sub-Committee is preparing for the PCIG workshop in Beijing at HTAi2020. It's co-lead, Sam Thomas, has had to step down due to other commitments. We're all enormously grateful to Sam whose time, energy and skills have been really valued by this project in addition to his other contributions to PCIG such as organising the PCIG workshop in Cologne and coordinating a recent PCIG consultation response. Thank you Sam.

Ann Single, Chair – HTAi Patient and Citizen Involvement Interest Group

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HTAi Matters



HTAi 2020 Annual Meeting Beijing
Attaining, maintaining and sustaining healthcare systems in a changing World: the role of HTA HTAi2020.org
[20-24 June](#)

Early registration opened mid-January 2020. Acceptance notifications for abstracts due February 1, 2020.

Travel grants <https://www.htai2020.org/travel-grants-2/>

Successful/Unsuccessful Application Notification: February 3, 2020

HTAi is aware of the global news regarding the Coronavirus (2019-nCoV) and the evolving advisories regarding travel to China. We will continue to closely monitor the situation and its impact on our stakeholders. We will provide updates on the Annual Meeting in the travel tab of the Annual Meeting website, found here: <https://htai2020.org/traveling-to-china/>.

We encourage you to check back periodically for the most recent information.

Policy Forum. New Orleans 26-28 January, 2020: Deliberative Processes in Health Technology Assessment: Prospects, Problems, and Policy Proposals

The background paper was developed by Ken Bond (Scientific Secretary, GPF) Dan Ollendorf (Chair, GPF) and Rebecca Trowman (HTAi Director, Scientific Initiatives) with the guidance of HTAi Global Policy Organizing Committee members.

The International Journal of Technology Assessment in Health Care (IJTAHC)

The journal has published the 2019 Annual Meeting Abstract Supplement, available [here](#).

The journal is seeking a Social Media Editor to promote the journal's public presence on social media. Cambridge University Press publishes this journal of the Society for Health Technology Assessment International – HTAi. This is an extremely important role which has the potential to have a major positive impact on IJTAHC. Cambridge University Press is looking for someone who is embedded in this discipline, with strong network links and abilities. They will have a high level of social media literacy. Specifically, Cambridge University Press seeks someone to manage a Twitter account on behalf of the journal. Students are encouraged to apply. Applicants are asked to indicate in their cover letter, specific actions that would be undertaken to promote IJTAHC, as well as provide evidence of their influence (via SoMe). Additionally, your understanding/experience of academic publishing and in particular of IJTAHC needs to be provided. Details of how you would approach this new role and your thoughts regarding evaluating the impact of this work should be included in your response. A small stipend is offered of \$500 USD annually, plus an additional \$500 USD worth of Cambridge books at the start of the engagement.

Please send a letter detailing your suitability for the role and a CV to Associate Professor Wendy Babidge: wendy.babidge@adam.com.au by 14 February 2020.

Follow us on social media and re-post our messages: #HTAi2020Beijing

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What's Happening

US FDA Oncology Center of Excellence (OCE)

February 5, 2020 FDA Oncology Center of Excellence (OCE) public meeting, the "[Envisioning Oncology Product Development for 2025](#)"!

The purpose of this meeting is to update the public on some of our major innovative programs at OCE. We'll also be celebrating the third year of OCE's operation

Registration is available online [here](#) to attend in-person or via WebEx. We are excited to provide an update on OCE programs and hear from you and other stakeholders regarding opportunities for collaboration and innovation in the future!

The official meeting hashtag for social media is #OCEProject2025. Follow @FDAOncology on Twitter and #OCEProject2025 for meeting updates.

The public meeting will be held at the FDA's White Oak Campus from 9:00 am – 1:00 pm. Please let us know if you require any further information or assistance. We sincerely hope to see you at the "[Envisioning Oncology Product Development for 2025](#)" on February 5th.

US FDA Center for Devices and Radiological Health (CDRH)

CDRH is hosting a co-sponsored summit with ISPOR on "Using Patient-Preference Information in Medical Device Regulatory Decisions: Benefit-Risk and Beyond" on March 31. ISPOR has just opened registration for in person and virtual attendance.

<https://www.ispor.org/conferences-education/conferences/upcoming-conferences/ispor-fda-summit-2020/about/registration-information>.

HealTAC 2020: April 23-24, 2020, London

<http://healtex.org/event/healtac-2020-save-date/>

The third HealTAC conference will be held in central London in April. HealTAC 2020 will bring the academic, clinical, industrial and patient communities together to discuss the current state of the art in processing healthcare free text and share experience, results and challenges.

Submissions: We invite full and short papers, posters, PhD student/early postdoc papers, demos and panel proposals that address any methodological or implementational aspect involved in processing and using healthcare free text. Submissions are welcome from all disciplines interested in this area (NLP, clinical and social care, legal, auditing etc.). The submission site is now open - please see the details [here](#).

Keynotes: The keynotes will be delivered by Prof Kalina Bontcheva (University of Sheffield) and Prof Patrick Ruch (HEG/HESSO Geneva and Swiss Institute of Bioinformatics).

Pre-conference event: We are also delighted to announce that there will be a pre-conference event on April 22nd about 'Online Life and Mental Wellbeing'. This will be an interactive workshop with invited speakers and themed discussion sessions. The event is open to the public but with limited spaces: the agenda and registration details will be announced soon.

Key dates:

January 20th, 2020: Deadline for full and short papers, and panel proposals

February 10th, 2020: Deadline for posters, PhD forum and demo proposals

February 23rd, 2020: Notification of acceptance

Follow further announcements at [#HEALTAC2020](#)

Submitted by Sharmila Sousa

You might remember that I was [in the UK parliament](#) last year to help members of parliament ask universities and hospital trusts why they were not following the rules on reporting results. Those of you who watched the parliamentary session saw the impact the questioning had. Because the politicians asked direct questions about specific unreported trials the university representatives were forced to give a detailed explanation for every missing result. They universally told the politicians, 'we are now planning to report this trial very soon.' It was highly effective!

Imagine the impact we could have if we made institutes across Europe feel under the same pressure to give an explanation for every unreported clinical trial. You can help us do that.

The politicians were armed with information from the EU TrialsTracker website. You can find and use information from there too. Identify an institute you are interested in – maybe you're a staff member or alumnus; or perhaps you volunteered for a clinical trial run by a university or hospital – and ask them to explain their plan to report results for their unreported trials. There is a complete [set of instructions](#) on identifying unreported trials and suggestions for what you should write on the AllTrials website at <https://www.alltrials.net/ask-your-institution/>

We think that putting rule breakers under this kind of scrutiny will get missing results reported. Let us know who you contact and if you get a response, and we will monitor the impact. We can't wait to hear how you get on.

Síle, Sense about Science www.senseaboutscience.org.

Rare Diseases Day 2020! <https://www.rarediseaseday.org/>

February 29th, 2020

Organised by EURORDIS <https://www.eurordis.org/>

Dutch hospital resumes production of rare drug after sourcing purer ingredients

<https://www.bmj.com/content/368/bmj.m401>

BMJ 2020; 368 doi: <https://doi.org/10.1136/bmj.m401> (Published 30 January 2020)

A university hospital in the Netherlands has resumed cost price production of orphan medicine chenodeoxycholic acid (CDCA), a primary bile acid used to treat the rare hereditary metabolic disorder cerebrotendinous xanthomatosis (CTX). The pharmaceutical compounding initiative at Amsterdam University Medical Centre, which was intended to ensure access to the drug, was launched in 2018 after manufacturers, Leadiant Biosciences, increased the price of its CDCA fivefold. Production was suspended after a Dutch Health Care Inspectorate investigation found that the CDCA that the hospital made contained trace amounts of impurities. Supply has resumed.

Publications

B O'Rourke, S Werkö, T Merlin, L Huang, T Schuller. The 'Top 10' Challenges for Health Technology Assessment: INAHTA Viewpoint. *International Journal of Technology Assessment in Health Care*, 1-4. doi:10.1017/S0266462319000825

CM Kieffer, AR Miller, B Chacko, AS Robertson. FDA reported use of patient experience data in 2018 drug approvals. *The Innov & RegSci* 2019, 1-8

Patient experience data (PED) refers to the systematic collection of meaningful data relating to the experiences, perspectives, needs, and priorities of patients. To augment clinical trial data in review of product applications 59 new molecular entities approved in 2018; 48 had a table summarising whether and if so how PED was used; 34 used PED in the drug review. PROs were the most significant source of PED (in 29 of 48 sponsor submitted, 9 specified the specific PRO)

FDA considered nonsponsor-submitted PED in 5 of 48 reviews: 4 from Patient Focused Drug Development (PFDD) meetings, 1 of patient stakeholder held meeting with FDA, 1 from Advisory Committee meeting (4 of the 5 were from ODEIII)

Submitted by Nigel Cook

JC Bouvy, L Cowie, R Lovett, D Morrison, H Livingstone, N Crabb, 2020. Use of Patient Preference Studies in HTA Decision Making: A NICE Perspective. *The Patient - Patient-Centered Outcomes Research*

<https://doi.org/10.1007/s40271-019-00408-4>

https://link.springer.com/epdf/10.1007/s40271-019-00408-4?shared_access_token=wzunJbW7l7qyL4epLU4TMPe4RwlQNchNByi7wbcMAY4hGNNlz6Nxnc-jS83-siyYdgCvbm5Brcial5BXCKNCKB4oM3KroGn8xtWH5wjFd73jAhzkewwbokWD2YAsq7JG66USbFKPl4sqO_OmrKFyQw%3D%3D

Patient preference studies could provide valuable insights to a National Institute for Health and Care Excellence committee into the preferences patients have for different treatment options, especially if the study sample is representative of the broader patient population. We identify three main uses of patient preference studies along a technology's pathway from drug development to clinical use: in early clinical development to guide the selection of appropriate endpoints, to inform benefit-risk assessments carried out by regulators and to inform reimbursement decisions made by health technology assessment bodies. In the context of the National Institute for Health and Care Excellence's methods and processes, we do not see a role for quantitative patient preference data to be directly incorporated into health economic modelling. Rather, we see a role for patient preference studies to be submitted alongside other types of evidence. Examples where patient preference studies might have added value in health technology assessments include cases where two distinctly different treatment options are being compared, when patients have to decide between multiple treatment options, when technologies have important non-health benefits or when a treatment is indicated for a heterogenous population.

J Carlton, T Peasgood, S Khan, R Barber, J Bostock, AD Keetharuth. 2020. An emerging framework for fully incorporating public involvement (PI) into patient-reported outcome measures (PROMs). *Journal of Patient-Reported Outcomes*, 4(1), 4. <https://doi.org/10.1186/s41687-019-0172-8>

IO Halabi, B Scholtes, B Voz, et al. 2020. Patient participation and related concepts : A scoping review on their dimensional composition. *Patient Education and Counseling*, 103(1), 5-14. <https://doi.org/10.1016/j.pec.2019.08.001>

R Kimerling, ET Lewis, SJ Javier, DM Zulman. 2020. Opportunity or Burden ? A Behavioral Framework for Patient Engagement. *Medical Care*, 58(2), 161-168. <https://doi.org/10.1097/MLR.0000000000001240>

C Roberts, H Rochford-Brennan, J Goodrick, et al. 2020. Our reflections of Patient and Public Involvement in research as members of the European Working Group of People with Dementia. *Dementia (London, England)*, 19(1), 10-17. <https://doi.org/10.1177/1471301219876402>

A Fabbri, L Parker, C Colombo, et al. 2010. Industry funding of patient and health consumer organisations: systematic review with meta-analysis. *BMJ*. 2020 Jan 22;368:l6925. doi: 10.1136/bmj.l6925. Free Article

Wilson MG, Nidumolu A, Berditchevskaia I. Identifying approaches for synthesizing and summarizing information to support informed citizen deliberations in health policy: A scoping review. <https://journals.sagepub.com/doi/10.1177/1355819619872221#articleShareContainer>
Journal of Health Services Research & Policy. 2019.

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