HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG)
E-Bulletin, July 2023

Enhanced quality and relevance of HTA through patient and citizen involvement

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

Welcome to our July E-Bulletin

Dear HTAi PCIG Members

Greetings to everyone and a hearty and warm welcome to our new members!
Your dedication to advancing patient and citizen involvement in healthcare and HTA is commendable, and we look forward to working together to make a positive impact.
We are thrilled to embark on this journey together as the PCIG co-chairs with our Vice-Chair Zal Press and the valuable assistance of our Technical Officer Hadewych Honné. Thank you for entrusting us with this responsibility.

We look forward to fostering a collaborative and innovative environment within the group.
With warm regards,
Aline Silva and Fiona Pearce

NOTE: Present and past issues of the E-Bulletin can also be accessed on the website
https://htai.org/patient-and-citizen-involvement/
Social media accounts on LinkedIn and twitter (@pcisg)

PCIG Matters

Does patient and public involvement impact public health decision-making? A 10 year retrospective analysis of public consultation in Brazil?
Ana from Conitec, Brazil shares a recent publication: De Freitas Lopes AC, Novaes HMD, De Soárez, PC. Does patient and public involvement impact public health decision-making? A 10 year retrospective analysis of public consultation in Brazil. Health Res Policy Sys 21, 72 (2023). https://doi.org/10.1186/s12961-023-01018-1
https://link.springer.com/article/10.1186/s12961-023-01018-1
The study analyzed all public consultations conducted by Conitec/Brazil on technology incorporation processes in the public health system during its first 10 years of operation. It was conducted by researchers from the University of Sao Paulo and me, from the Executive Secretariat of Conitec, as first author.
As a more relevant result, I would like to point out that the greater participation of patients and family members was significantly associated with changing Conitec's recommendation, which reinforces the importance of involving patients and public in HTA decision-making.

Contributed by
Ana Carolina de Freitas Lopes

HTAi Matters

HTAi 2023 awards
The South African Values and Ethics (SAVE) UHC methodology was the winner of the HTAi Egon Jonsson award for most outstanding paper of the year. The paper describes developing and piloting a context-specified ethics framework for health technology assessment.
Krubiner C, Barsdorf, N, Goldstein S, Mosam A, Potgieter S, DiStefano M, . . . Hofman K (2022). Developing and piloting a context-specified ethics framework for health technology assessment: The South African Values and Ethics for Universal Health Coverage approach. International Journal of Technology Assessment in Health Care, 38(1), E26. doi:10.1017/S0266462322000113

Best Reviewer Award, IJTAHC Editorial Board
Professor Tracey Sach, United Kingdom
Special Mention:
Clifford Goodman, United States
Karen Facey, United Kingdom
Ken Bond, Canada

The HTAi Review your biweekly news source for all things HTAi at https://htai.org/htai-review
to catch up on HTA news and updates from around the world.

Website: https://htai.org/

HTAi Social Media
Feel free to follow us or check in on our social media channels and re-post our messaging:
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What's Happening

Public consultation on WHO guidance for best practices for clinical trials
https://www.who.int/news-room/articles-detail/public-consultation-on-who-guidance-for-best-practices-for-clinical-trials
In May 2022, the Seventy-fifth World Health Assembly adopted a resolution on Strengthening clinical trials to provide high-quality evidence on health interventions and to improve research quality and coordination, in which one action requested of the Director-General was to develop WHO guidance on best practices for clinical trials.
Important stakeholder groups for this technical guidance include, (but are not restricted to): public sector researchers, private sector entities engaged in clinical trials, national health authorities or research councils involved in health research, clinical trial registries, research ethics bodies, national or transnational medicinal product regulatory authorities, decision-making bodies making use of evidence such as guidelines developers, and health technology assessment bodies, healthcare practitioners, patient engagement and community engagement entities, and professional associations in disciplines for whom clinical trials of health interventions are relevant. There may also be some relevance to medical journals.
We would like to receive your overall comments on what the draft guidance does well and less well. Please use the online form accessible through this link to provide inputs.

DEADLINE for the submission of comments is 15th September 2023. Please feel free to contact us at WHA758@who.int for additional information.
Please note we are providing a Word file of the full list of questions to help you plan your online submission

Theme Announced for ISPOR Europe 2023, Copenhagen 12-15 November
https://www.ispor.org/conferences-education/conferences/upcoming-conferences/ispor-europe-2023/program/program/?utm_medium=email&utm_source=database&utm_campaign=ispor_europe_2023&utm_content=ispor_europe_2023_program_launch_july24&_zs=3hXOX&_zl=bfFg3
The conference theme is on health economics and outcomes research (HEOR): 'HEOR at the Nexus of Policy and Science'. This topic, and many others, will be featured during expert-led science and policy sessions at the conference to be held in Copenhagen, Denmark. View the Schedule-at-a-Glance, explore sponsorship and exhibitor opportunities, and learn more and register here.
Travel support is available through ISPOR's Travel Grant program to support attendance. Eligible members, students, and patient representatives are encouraged to apply by 28 August.

New this year: Enhance your conference registration by adding the new Digital Conference Pass! Access recordings of nearly all of the educational sessions, plenaries, spotlights, and other content from the conference, plus a post conference Key Insights Session exclusive to Digital Conference Pass holders that will recap and comment on all of the activities that took place in Copenhagen
Register by 5 October to save up to 20% with our Early Bird rates!

ICER Launches a Patient Council to Amplify the Patient Voice in Value Assessment
https://icer.org/news-insights/press-releases/icer-launches-a-patient-council-to-amplify-the-patient-voice-in-value-assessment/
The US Institute for Clinical and Economic Review (ICER) has announced the creation of a Patient Council to advise on ICER's patient engagement strategy, outreach, and process for input into drug reviews and broader initiatives. The Patient Council will advise ICER on engaging with patient groups while also identifying strategies to communicate ICER's work to patient communities and the broader public.

Five leading agencies collaborate to promote Early HTA for development of medical and healthcare innovations in Thailand
https://www.hitap.net/en/news/186699
On June 19, 2023, the Thai Health Intervention and Technology Assessment Program Foundation (HITAP Foundation), in collaboration with the Saw Swee Hock School of Public Health of the National University of Singapore (NUS), organized a workshop on 'Fruitful Research: How to Make Health Innovation Worthwhile'. Together with partner agencies, including the Thailand Science Research and Innovation (TSRI), the Thailand Center of Excellence for Life Sciences (Public Organization) or TCELS, and the National Health Security Office (NHSO), they also signed a Memorandum of Understanding (MOU) on "Collaboration on Priority Setting and Early Health Technology Assessment of Health Innovations in Thailand". In the workshop, Dr Yot Teerawattananon, Secretary General of the HITAP Foundation, and Dr Wang Yi, Assistant Professor at Saw Swee Hock School of Public Health, National University of Singapore, shared their knowledge and experience about early HTA, covering topics such as, what makes a health innovation 'desirable' and 'marketable', and how to know if it is worth investing in research and development of a certain health innovation.

EMA publishes review of its studies on use of real-world evidence in regulatory decision making
https://www.ema.europa.eu/en/news/use-real-world-evidence-regulatory-decision-making-ema-publishes-review-its-studies
Real-world evidence (RWE) from studies led by regulators can complement evidence from other sources including clinical trials. RWE can support both pre-authorisation and post-approval assessments of EMA's scientific committees, working parties and national competent authorities. However, more effort is needed

to better anticipate the need for such studies and to speed up their initiation to ensure that regulators have access to RWE in a timely manner.
These are some of the findings of a report published today on the experience EMA has gained in conducting studies with real-world data (RWD) in the past year and a half. The report is part of the Agency's efforts, alongside the European Medicines Regulatory Network (EMRN), to enable use of RWD in regulatory decision-making.
In pharmacovigilance, it has become mainstay to use routinely collected data about a patient's health status or the delivery of healthcare from a variety of sources other than traditional clinical trials to support decision-making. However, the use of RWE is less established in earlier stages of medicines development.
Overseen by the EMA-HMA Big Data Steering Group, EMA and EMRN are working towards setting up a sustainable framework that enables the use and establishes the value of RWE in decision-making across the entire product lifecycle.
The report covers the period from September 2021 until the first anniversary of the Data Analysis and Real-World Interrogation Network DARWIN EU® on 7 February 2023. During this period, 61 RWD research opportunities were identified, 30 studies initiated and 27 completed.
Real-world evidence framework to support EU regulatory decision-making: Report on the experience gained with regulator-led studies from September 2021 to February 2023 (PDF/1.92 MB) (updated)

How Knowledge Graphs Help Pharma Companies With Data Visualization (IQVIA)
https://www.iqvia.com/blogs/2023/06/how-knowledge-graphs-help-pharma-companies-with-data-visualization
Globally, regulatory agencies are asking pharma companies to use real-world data and real-world evidence (RWE) in their decision making across drug discovery, development, and commercialization. One pharma company applied innovative technologies to identify the stakeholders across the RWE landscape to help understand digital health data policies and pinpoint the key opinion leaders and influencers in this space. IQVIA experts in regulatory science and strategy and real world data worked with IQVIA technical experts to use a combination of AI technologies (natural language processing and knowledge graph) to create an effective robust solution. Knowledge graphs are conceptual models of various entities and their relationships within a network that enables users to quickly find and connect heterogenous data by means of standardized ontologies.

The National Health Service (NHS) in the UK has ring-fenced funding to enable faster diagnosis of conditions such as cancers, strokes and heart conditions
https://businesscloud.co.uk/news/saving-brains-21m-fund-to-roll-out-ai-across-nhs/
A newly announced fund will help implement artificial intelligence (AI) in NHS Trusts. The AI Diagnostic Fund will help promote the use of AI solutions for imaging and diagnostics for conditions like cancer, cardiovascular disease, and stroke. AI tools will be used to analyse chest X-Rays – the most common tool used to diagnose lung cancer. The use of AI in the NHS is already having a positive impact on outcomes for patients, with AI in some cases halving the time for stroke victims to get the treatment they need by helping doctors diagnose stroke faster, which has been shown to triple the chance of patients living independently after a stroke.

One-stop-shop for AI and digital regulations for health and social care launched
https://www.nice.org.uk/News/Article/one-stop-shop-for-ai-and-digital-regulations-for-health-and-social-care-launched
A new online advice service has been launched to help the NHS and wider care system adopt and make use of new digital and artificial intelligence. It provides useful and useable guidance on how to identify, pilot and rollout such technologies. This service is a multi-agency collaboration between NICE, the Care Quality Commission, the Health Research Authority and the Medicines and Healthcare products Regulatory Agency. The AI and Digital Regulations Service provides guidance for NHS and social care adopters and

digital health innovators.

GIN 2023
https://g-i-n.net/conference_2023/welcome
This year's Guidelines International Network (GIN) conference takes place from Tuesday 19 to Friday 22 September 2023 in Glasgow and online. The theme is 'Maintaining quality while pursuing efficiency'.

Sun rises on Dawn Health, Novartis' health-tracking app for multiple sclerosis
By Andrea Park https://www.fiercebiotech.com/medtech/sun-rises-dawn-health-novartis-health-tracking-app-ms
The app Ekiva-MS works by collecting a variety of health and behavioral data that users can then review to better understand how MS impacts their lives. The app aims to help people with MS to better understand the disease and, in doing so, improve their care. This is the first result of the partnership between Novartis and Danish health-focused software developer Dawn Health. The goal is to create a software platform equipped with tech tools that'll help individuals manage a wide range of chronic diseases.
The duo was able to launch their first offering within six months by relying both on close collaboration with actual MS patients and on their respective expertise—Novartis' wealth of disease research and work in digital health, and Dawn's development of digital therapeutics and other software-as-a-medical-device tools. The app collects those data in multiple ways: by asking users to manually track changes in their symptoms and overall quality of life, and by using their smartphones' built-in sensors to capture other day-to-day information.

FDA Center for Biologics Evaluation and Research Chief Marks overrules staff drug reviewers 'with trepidation'
https://www.fiercebiotech.com/biotech/fdas-marks-i-contradict-fda-reviewers-trepidation
The FDA granted accelerated approval to Sarepta's gene therapy for four and five-year-old patients with Duchenne muscular dystrophy. The decision was at odds with agency reviewers who said ahead of an advisory meeting to discuss the treatment that the available data did not provide "unambiguous evidence" of clinical benefit. "…it's important for us—for the community to know—that we're not so dead set on getting it right 100% of the time that we actually prevent things from getting to market that could potentially help people a bit sooner."
He would never overrule reviewers if there were questions about safety. Duchenne advocates who spoke at advisory hearings for the therapy were emphatic that the side effects were dwarfed by the risk of disease progression.

Johnson & Johnson (J&J) to allow a generic version of its life-saving tuberculosis (TB) drug bedaquiline to be supplied in many low- and middle-income countries
https://www.science.org/content/article/major-drug-company-bends-battle-over-access-key-tb-treatment
Patient advocacy groups had long called for the company to put an end to its 'patent evergreening', whereby it extends the patent protection period by holding patents on tweaks to the drug's formulation. In a deal with the Global Drug Facility (GDF), J&J has agreed not to enforce some of its patents in 44 countries, which could cut the drug's price by at least two-thirds. However, the deal excludes a large number of countries with a high TB burden, including Russia, where a local company has exclusive commercial rights to supply the drug, and South Africa and Indonesia, which don't procure drugs through the GDF.

The R&D landscape for infectious disease vaccines
https://www.nature.com/articles/d41573-023-00119-4?utm_source=Nature+Briefing%3A+Translational+Research&utm_campaign=89b7cf8e10-briefing-tr-20230726&utm_medium=email&utm_term=0_872afe2a9a-89b7cf8e10-47439240
Almost 1,000 vaccine candidates are currently being tested around the world. Recombinant-protein vaccines account for the largest proportion; they are popular because they are generally safe, stable and

easy to manufacture. Nucleic-acid vaccines, which include RNA and DNA vaccines, are now the second most common, thanks to the success of mRNA vaccines against COVID-19. Only 23% of current vaccine candidates are conventional inactivated or attenuated vaccines. Although a large chunk of vaccine development is dedicated to SARS-CoV-2, with at least 14 nasal vaccines alone in the works, there are also efforts to address non-viral pathogens such as malaria (57 candidates) and pneumococci (40 candidates). Yue J et al./Nat. Rev. Drug Discov July 2023)

Publications

Hogervorst M, Vreman R, Heikkinen I, Bagchi I, Gutierrez-Ibarluzea I, Ryll B, . . . Oortwijn W (2023). Uncertainty management in regulatory and health technology assessment decision-making on drugs: Guidance of the HTAi-DIA Working Group. International Journal of Technology Assessment in Health Care, 39(1), E40. doi:10.1017/S0266462323000375

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Aikaterini Fameli, Thomas Paulsson, Shannon Altimari, Ben Gutierrez, Ali Cimen, Linda Nelsen, Nick Harrison.
https://www.ispor.org/publications/journals/value-outcomes-spotlight/vos-archives/issue/view/looking-beyond-survival-data-in-oncology-reimbursement-decisions/looking-beyond-survival-data-how-should-we-assess-innovation-in-oncology-reimbursement-decision-making?utm_medium=email&utm_source=vos&utm_campaign=value_and_outcomes_spotlight&utm_content=vos_gsk_suppl_article&utm_term=vos_gsk_suppl_article&_zs=3hXOX&_zl=avEf3
Looking Beyond Survival Data: How Should We Assess Innovation in Oncology Reimbursement Decision Making
In some treatment settings, reliance on overall survival presents limitations in health technology assessment decision making. In the absence of overall survival, there is a need to better recognize the value of alternative endpoints and patient-reported outcomes, either as predictors of survival and/or measures of stand-alone value. Stakeholders need to work together to support greater use of alternative endpoints and patient-reported outcomes in decision making to ensure future health technology assessments result in the best outcomes for patients.

Chris J Edgar, Elizabeth (Nicki) Bush, Heather R Adams, Rachel Ballinger, Bill Byrom, Michelle Campbell, Sonya Eremenco, Fiona McDougall, Elektra Papadopoulos, Ashley F Slagle, and Stephen Joel Coons
This report presents emerging good practice in the development, selection, and modification of performance outcome assessments, including the evaluation and documentation of validity, reliability, usability, and interpretability evidence.
Recommendations on the Selection, Development, and Modification of Performance Outcome Assessments: A Good Practices Report of an ISPOR Task Force
https://www.ispor.org/publications/journals/value-in-health/abstract/Volume-26--Issue-7/Recommendations-on-the-Selection--Development--and-Modification-of-Performance-Outcome-Assessments--A-Good-Practices-Report-of-an-ISPOR-Task-Force?utm_medium=email&utm_source=database&utm_campaign=value_in_health&utm_content=value_in_health_july_etoc_july_14&utm_term=recommendations_on_the_selection,_development,_and_modific

ation_of_performance_outcome_assessments:_a_good_practices_report_of_an_ispor_task_force&_zs=3hXOX&_zl=iJaf3

Lakin K, Kane S. What can one legitimately expect from a health system? A conceptual analysis and a proposal for research and action. BMJ Global Health 2023;8:e012453. http://dx.doi.org/10.1136/bmjgh-2023-012453 https://gh.bmj.com/content/8/7/e012453

Erku D, Walker D, Amaris A, Wubishet B, Assefa Y, Abera S, . . . Scuffham P (2023). Institutionalizing health technology assessment in Ethiopia: Seizing the window of opportunity. International Journal of Technology Assessment in Health Care, 1-12. doi:10.1017/S0266462323000454

Saygın Avşar, Hasan Hüseyin Yıldırım National Health Technology Assessment in Turkiye after a decade: Are key principles followed? International Journal of Technology Assessment in Health Care
doi: 10.1017/S0266462323000466

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