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

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 June E-Bulletin

Introduction to our new Co-Chairs, Vice Chair, and Steering Group member!

This E-Bulletin closely follows the completion of the HTAi Annual Meeting in Adelaide, Australia and I have stepped in to introduce our new leaders, only from what I know about them. The notes on the Annual Meeting are brief as we participants continue to absorb our experiences.

Our Co-Chairs are 'people of the world' who move between countries in their work with patient advocates. Aline Silveira Silva originally worked in Sciences and Health Technologies for the Ministry of Health in Brazil, is now at the University of British Columbia, Vancouver, has lived experience of breast cancer and is involved with Patient Voices Network, Canada.
Fiona Pearce is an Australian who attended university in Australia and is now Senior Advisor to the Agency for Care Effectiveness (ACE), HTA and Consumer Engagement & Education teams, Ministry of Health in Singapore. She has ably developed and presented comprehensive resources for patient involvement in HTA in Singapore. This is something the PCIG has worked on for many years, and so her experience will provide invaluable guidance for a new defined project to review PCIG resources.
https://www.ace-hta.gov.sg/Patients-And-Community/opportunities-for-patient-involvement?utm_source=LI&utm_medium=Social&utm_campaign=LI_Process_Guide_for_patient_involvements_ST
Our new Vice Chair is Zal Press, Vice-Chair or the CADTH Patient and Community Advisory Committee in Canada and he is Founder and Executive Director of Patient Commando. This an independent patient group dedicated to amplifying the patient voice in all channels of the delivery of care.
We also have a new Steering Committee member, Catherine Koola, who is Associate Director of Patient Engagement, Institute for Clinical and Economic Review (ICER) in the USA.

Aline was at the HTAi Annual Meeting in Adelaide, Australia and ably took over the moderation of the PCIG Annual Business Meeting. This meeting was well attended and noisy with much going on. Many of the people we wanted to connect with, stimulated by events at the conference, were in the same room.
Pleasant news is that Veronica Gousset, our previous enthusiastic and hard-working Technical Officer, has given birth three weeks early to baby Zoe. Welcome Zoe Gousset and congratulations Veronica and Arthur.

HTAi2024 will be in Seville, Spain and Steering Committee member Ana Toledo Chavarri is on the International Scientific Program Committee for this Annual Meeting.

Thankyou Ann Single for all your hard work for the PCIG over the years and we wish you every success as Vice-President of HTAi.

Janney Wale, Editor of the PCIG E-Bulletin

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

PCIG Annual Business Meeting, held Tuesday 27 June 2023
Current membership (June 2023): 305
To:
Promote and develop robust methodologies
Share best practice in participation
Strengthen HTA by systematic incorporation of patient & citizen perspectives
Support countries with limited experience

The new Steering Committee is:
Aline Silveira Silva: Co-Chair (Patient Voices Network, Canada, University of British Columbia, Vancouver, Canada)
Fiona Pearce: Co-Chair (Senior Advisor to the Agency for Care Effectiveness (ACE), Ministry of Health in Singapore)
Zal Press: Vice-Chair (CADTH Patient and Community Advisory Committee, Patient Commando)
Ann Single, Outgoing Chair (Patient Voice Initiative (PVI), Australia)
Anke-Peggy Holtorf, Project Co-ordinator (Health Outcomes Strategies, Switzerland)
Neil Bertelsen (nb consulting, Germany)
Todd Stephenson, Financial Secretary (Janssen, Australia)
John Gillespie (Abbott, Asia-Pacific)
Barry Liden (USC Schaeffer & Patient Voice Advisors, USA)
Heidi Livingstone (NICE, UK)
Sarah Berglas (CADTH, Canada)
Ana Toledo Chavarri (Canary Islands Health Service, Spain)
Catherine Koola (Associate Director of Patient Engagement at the Institute for Clinical and Economic Review (ICER), USA)
Kate Morgan (Myeloma Patients Europe, UK)
Valentina Strammiello (European Patients Forum, Belgium)

Technical Officer: Hadewych Honné (PhD student, Edinburgh University & KU Leuven)
Project Co-Ordinator, Anke-Peggy Holtorf

Past Chairs
Karen Facey (Founder): 2005-2011; Janney Wale: 2011- 2016; Neil Bertelsen: 2016-2019; Ann Single: 2019-2023

HTAi2023 Annual Meeting – a strong consumer and patient advocacy presence
The strong presence was noted and commended by many of the attendees at the Annual Meeting. Overall, 49 patient advocates were supported through the PCIG to attend (12 x PCIG PASS + 5 HTAi Grants + 32 local Australian PVI Grants).

Other Australian consumer advocates including from the Australian Department of Health and Aged Care HTA committees also attended.
One of the latter consumer advocates, Jack Nunn, writes:
"It was inspiring to attend my first HTAi conference, especially after having to miss out last year. It was a fascinating mix of people, patients, researchers, economists, assessors, policy makers and people from various industries. My overriding impression was of a strong international movement to improve how we assess health technology, and a strong desire to improve how we involve all stakeholders in doing that - most importantly - us - the public. It was also strangely reassuring to see that no one country seemed to have worked out the perfect HTA process, each country seeming to do different things well, with opportunities for learning in all directions. It was also reassuring to meet 'experts' just as confused as me by questions such as 'who decides what is ethical', and 'what does evidence informed stakeholder involvement look like?'.
For me the real highlight was seeing old friends, making new ones - and connecting with the other people around the world who work to give a voice to the public, patients and users of health technology. It was inspiring to attend the PCIG Annual Business Meeting and see all the dedicated people around the world volunteering to make this area of work more accessible, understandable and effective. I look forward to getting more involved, and perhaps even finding others interested in working with me on creating standardised ways to report HTA, including stakeholder involvement by using Standardised Data on Initiatives (STARDIT). Thank you to everyone who helped make it such a fantastic conference."

The PCIG thanks: the following companies for their generous support which provided PCIG PASSes for 12 patients and patient representatives (from outside Australia) to attend Annual Meeting for free: Sanofi, Janssen, Intuitive. Plus support from Roche
The PCIG worked with Patient Voice Initiative (PVI, Australia) who offered a PVI Grant for 32 people based in Australia, with the generous support of: Alexion, Amgen, AstraZeneca, Bristol Myers Squibb, CSL Vifor Pharma, Janssen, Lilly, Pfizer, Roche, Sanofi, Takeda.

One of the PCIG projects is to support 'Patient & public involvement at HTAi Annual Meeting' (#012)
Outcomes
Guidance for HTAi on including patients at HTAi Annual Meeting
Consistent with Patients Included Conference framework
Offering what is Essential and Beneficial to suit a range of hosts
Annual Meeting guidelines - addendum
The HTAi Secretariat have worked hard to meet guidance (including offering travel & accommodation arrangement for PCIG PASS recipients in 2023) and establishing a sponsored Patient (Access) Lounge
Project members: Ann Single (lead), Sarah Berglas (CADTH, Canada) David Boman/Andrew Zebrak (Intuitive) David Chandler (lay member UK), Linda Daniel (Patient Federation, Netherlands), Cor Oosterwijk (patient representative, 2022 Local Organising Committee), Paula Orecklin (patient member, Canada), Valentina Strammiello (European Patients Forum, Belgium) Dorota Zgodka (independent consultant).

Patient and Citizen Involvement in HTA workshop, June 24
After a morning highlighting PCIG projects and the work of key international players, there were breakout groups with three streams to choose from:
Asia Pacific 360: What's currently happening in patient involvement in HTA in the Asia Pacific, what goals do we have, can we work together to document practice and describe good practice?
Training: What's currently being offered to patient communities, where are the synergies, where are the gaps, how should this inform PCIG's review of its tools and resources?
Patient Preferences: How are patient preference studies currently informing HTA, how can we work together to improve their integration.

Session Summary - Enriching Healthcare Evidence Generation and Decision-Making: Integrating Patient Experience Data (PED) and Patient Engagement (PE) has been linked to HTAi PCIG by Synapse.
https://patientengagement.synapseconnect.org/resources/session-summary-enriching-healthcare-evidence-generation-and-decision-making-integrating-patient-experience-data-and-patient-engagement
The session was moderated by Neil Bertelsen and consisted of presentations by Juan García Burgos representing EMA, Robyn Bent representing FDA, Rebecca Vermeulen representing Roche, and discussing industry efforts with PE and PED, Denis Costello, representing CML Advocates Network discussing the importance of patient community engagement.

HTAi Matters

HTAi 2023 Annual Meeting, June 24 to 28, Adelaide: The Road to Policy and Clinical Integration
https://htai.eventsair.com/htai-23-adelaide-am/main-theme
Plenaries:
Fast-Tracking Clinical Innovation: The Balance of Speed and Rigour – a 'hypothetical' around Alzheimer's disease
Making HTA More Efficient: What Can we Learn about Harmonization, Work Sharing, and Adaptation?
Feasibility of Aligning Technology Evaluation Processes and Decisions in an Era of Sustainable Development
Symposia:
Precision Oncology: Aligning innovation & Assessment to address patient access challenges
Value creation and efficiency gains through capacity-enhancing innovations to address workforce shortages
From Data to Health: Enhanced evidence methods and practical applications for HTA and treatment access in prostate cancer
Counting the cost – assessing the true value of investing in cancer treatments
- These gave good broad perspectives from a range of experts always including patient advocates.

The Patient Lounge at the conference centre successfully facilitated a patient-friendly environment to improve the overall experience of patient delegates.

The HTAi Review your biweekly news source for all things HTAi at https://htai.org/htai-review-june-9-2023/ 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:
Twitter: @HTAiOrg
Facebook: @HTAiOrg
LinkedIn

What's Happening

The EUnetHTA 21 Consortium Will Cease Operations on 16 September 2023
https://www.eunethta.eu/the-eunethta-21-consortium-will-cease-operations-on-16-september-2023/
The European Health and Digital Executive Agency signed the Service Contract for the Provision of Joint Health Technology Assessment (HTA) Work Supporting the Continuation of EU Cooperation on HTA (CHAFEA/LUX/2020/OP/0013) with the EUnetHTA 21 Consortium. Continued collaboration will be facilitated under the European Regulation on HTA, with the HTA Coordination Group supporting its continued implementation.

European Countries Flag Four Medicines for Joint HTA Evaluation (The Pink Sheet)
https://pink.pharmaintelligence.informa.com/PS148197/European-Countries-Flag-Four-Medicines-For-Joint-HTA-Evaluation
The European cross-country coalition BeNeLuxA wants to conduct a joint health technology evaluation on drugs for treating Duchenne muscular dystrophy and amyotrophic lateral sclerosis, among others.
By January 2025, all oncology products, advanced therapy medicinal products (ATMPs) and medical devices will be mandated to undergo joint clinical assessment (JCA), with extension for all orphan drugs in 2028.

European Patients Forum (EPF) Newsletter
EPF's Julie SPONY and Valentina Strammiello travelled to Australia for Health Technology Assessment international (HTAi)'s Annual Meeting – Valentina as then Vice-Chair of PCIG Steering Committee expertly moderated the PCIG workshop on the Saturday.

The EU4Health programme is a unique opportunity to make a significant contribution to stronger, patient centred health systems that deliver for all, in all circumstances. EPF calls for a patient-centered 2024 work programme and has identified three areas for stronger action:
 A sustainable, accessible, and transparent funding scheme for patients and civil society organisations is essential to ensure that patient organisations can play their role in the health programme and other European legislation.
 Capacity-building programmes for patient participation in EU and national health policies: patients must have the necessary awareness of policy processes, training, and resources to participate in expert groups and advisory bodies.
 Substantial investment in health literacy and patient information. Health literacy fosters access to health and puts citizens and patients at the centre of health and healthcare.
Read the entire position statement here.

Impact of Multiple Sclerosis Symptoms (IMSS) Survey is LIVE
The European Multiple Sclerosis Platform (EMSP) is leading, alongside 25 MS Societies across Europe, on a pan-European survey to understand in depth the symptoms of Multiple Sclerosis and their impact on the quality of life, called IMSS, English abbreviation for Impact of Multiple Sclerosis Symptoms. The survey will be running until end of July 2023.
This information is needed to help us understand MS symptoms and their impact to prioritize symptom management and the need for proper, personalized, accessible, and timely care for MS symptoms.
Who can participate? People living with MS in the following countries can participate: Belgium, Croatia, Czech Republic, Denmark, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Netherlands, Norway, Poland, Portugal, Romania, Serbia, Slovenia, Spain, and Switzerland.
The IMSS survey is available here.

EUPATI: Registrations for the 2023 - 2024 Patient Expert Training Programme now open
The training is about the medicines development process and tailored for patients and patient

representatives (e.g. caregivers and people working in patient organisations).
Access information on the entire EUPATI Patient Expert Training Programme here.
Download their new programme brochure here.

US regulator approves Duchenne muscular dystrophy (DMD) gene therapy
https://www.nature.com/articles/d41573-023-00103-y?utm_source=Nature+Briefing%3A+Translational+Research&utm_campaign=812cd68462-briefing-tr-20230628&utm_medium=email&utm_term=0_872afe2a9a-812cd68462-47439240
The US Food and Drug Administration has granted accelerated approval for a gene therapy. The agency gave the green light to Sarepta's delandistrogene moxeparvovec for treating DMD. The approval came despite objections by review committees that were overruled by the head of the FDA's Center for Biologics Evaluation and Research. The therapy is approved for patients aged 4-5 years, and will be the second-most expensive therapy to reach the market, with a price tag of US$3.2 million.
https://www.nature.com/articles/d41586-023-01799-z?utm_source=Nature+Briefing&utm_campaign=cadab83fe1-briefing-dy-20230605&utm_medium=email&utm_term=0_c9dfd39373-cadab83fe1-44432605
What might convince FDA advisors is its efficacy in children under six: their muscle function significantly improved. "It's a vote for hope," says Donald Kohn, a stem-cell researcher and member of the FDA advisory committee.

Advancing Use of Patient Evidence in Decision-Making
Magda Chlebus, Jan Frich, Jan Geissler, Juan Garcia Burgos, Virginie Hivert, Álmath Spooner
https://globalforum.diaglobal.org/issue/june-2023/advancing-use-of-patient-evidence-in-decision-making/?utm_source=db&utm_medium=email&utm_campaign=global_forum&utm_content=PUB_GF_June_2023-06-10_nonmembers&mkt_tok=MzQ5LVNWSiowNjgAAAGMQLvv1y1wbJVSLTcEXV1t9bLf-AU8hUp2EhInTzRAccqL0iGq4Hti8z4yNSMxA0JnUQDlgfYk_nlg1Ye8SML3Eaj4KT_22E1saiTBNHnnWPc
The task of promoting the generation and use of meaningful patient-generated data in drug development is a shared one. An interactive DIA Europe 2023 session concluded that collaborations between regulators, industry, academia, and patient advocates will drive patient-focused medicines development.
The European Medicines Agency (EMA) has indicated that it wants robust patient experience data (PED) to be part of the marketing authorization process, and researchers and patients stand ready to embrace this opportunity. While several challenges remain to be addressed—notably in the areas of guidance, terminology, incentives, predictability, and transparency—the momentum is moving PED toward the center of decision-making. Industry, regulators, HTA bodies, research foundations, and patient advocates all play their part in catalyzing progress.
Inspired by a session at DIA Europe 2022, our diverse group of stakeholders has published a seven-point Patient Engagement Action Plan. In September 2022, the EMA held its workshop on Patient Experience Data in EU Medicines Development & Regulatory Decision-Making. Among the key outcomes of the EMA event was a commitment to produce a reflection paper that would advise on the generation and collection of PED.
Through scientific advice mechanisms and the qualification of new methodologies, European regulators can engage with investigators to support the development of high-quality patient evidence. The patient voice should be heard in developing scientific advice, evidence-generation plans, and regulatory assessment to ensure that they generate meaningful PED. Positive lessons can be learned from the pilot phase of the EMA's CHMP (Committee for Medicinal Products for Human Use) early contact with patient and consumer organizations.

Regulatory Reliance in Action Making a Difference for Patients - Regulators and Industry Come Together to Discuss Practical Solutions
Angelika Joos, Susanne Ausborn

https://globalforum.diaglobal.org/issue/june-2023/regulatory-reliance-in-action-making-a-difference-for-patients/?utm_source=db&utm_medium=email&utm_campaign=global_forum&utm_content=PUB_GF_June_2023-06-10_nonmembers&mkt_tok=MzQ5LVNWSiowNjgAAAGMQLvv1gpu03KG6mmzd_A54kIJ20ZkHJbGAN9sFan8rFl4UmyR-Q1xC54f65S5dHEaDSAgfPqF7UVn-x6I6XQQ2x_00BbzDu0M5VNFjaBfjx0
Senior regulatory experts representing health authorities from all around the world joined industry representatives at a DIA Europe 2023-affiliated workshop to discuss more effective and efficient information sharing to enable risk-based reliance reviews, the interpretation of product sameness, and the impact of the diverging country-specific requirements which are not necessarily justified from the point of view of modern regulatory science.
Benchmarking data collected annually by CIRS shows how submission delays and long assessment timelines are delaying the availability of new medicines for patients around the globe.
Reliance is the act whereby the regulatory authority in one jurisdiction takes into account and gives significant weight to assessments performed by another regulatory authority or trusted institution, or to any other authoritative information, in reaching its own decision. The relying authority remains independent, responsible, and accountable for the decisions taken, even when it relies on the decisions, assessments, and information of others.
https://www.who.int/publications/i/item/55th-report-of-the-who-expert-committee-on-specifications-for-pharmaceutical-preparations

An Interview With the Food and Drug Administration About Draft Patient-Focused Drug Development (PFDD) Guidance 3: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments
Elisabeth Oehrlein, Robyn Bent, et al. Value in Health 2023;26(6):791–795.
https://www.valueinhealthjournal.com/article/S1098-3015(23)02563-9/fulltext

The Guidance builds upon the 2009 patient-reported outcome (PRO) guidance, recognizing that guidance was needed not just for PRO measures but for all types of measures meant to reflect how patients feel, function, or survive. This required thinking about a framework that was relevant across a broad range of measures. Additionally, significant methodologic advances in the field of health measurement have been published in the years since the 2009 guidance, and the new guidance expounds on flexible options for clinical outcomes assessment (COA) development. Different types of validity are replaced with types of validity evidence and the presentation of that evidence in an explicit rationale to support whether the COA is fit-for-purpose. Both ideas arose from advances in validity theory.
Once all 4 of the PFDD guidances are final, they will replace the 2009 PRO guidance.

Guidance for industry: patient-reported outcome measures: use in medical product development to support labeling claims. (Food and Drug Administration)
http://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims
Patient-focused drug development: selecting, developing, or modifying fit-for-purpose clinical outcome assessments.
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-selecting-developing-or-modifying-fit-purpose-clinical-outcome
Patient-focused drug development guidance series for enhancing the incorporation of the Patient's voice in medical product development and regulatory decision making.
https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical
Principles for selecting, developing, modifying, and adapting patient-reported outcome instruments for use in medical device evaluation: guidance for industry and Food and Drug Administration staff, and other stakeholders.

https://www.fda.gov/regulatory-information/search-fda-guidance-documents/principles-selecting-developing-modifying-and-adapting-patient-reported-outcome-instruments-use

WHO and Republic of Korea sign landmark agreement to boost biomanufacturing capacity, 26 May 2023
https://www.who.int/news/item/26-05-2023-WHO-and-Republic-of-Korea-sign-landmark-agreement-to-boost-biomanufacturing-capacity
WHO and the Republic of Korea have signed a Memorandum of Understanding to establish a global training hub in biomanufacturing, to serve all low- and middle-income countries (LMICs) to produce biologicals, such as vaccines, insulin, monoclonal antibodies, and cancer medicines.
In parallel, WHO is intensifying regulatory system strengthening to ensure the quality and safety of health products. Stronger regulatory agencies will also enhance confidence in locally produced products and counter misinformation and the availability of unsafe substandard or falsified medicines.

Testing could help prevent further strokes in people with gene variant
https://www.nice.org.uk/news/article/testing-could-help-prevent-further-strokes-in-people-with-gene-variant
People who have had an ischaemic stroke or transient ischaemic attack (TIA) should have a genetic test to find out whether they can be treated with a drug which reduces the risk of further strokes. New draft guidance from NICE recommended clinicians offer laboratory-based genotype testing to people if treatment with the drug clopidogrel is being considered. This treatment is not suitable for those with certain variations in a gene called CYP2C19 because they cannot convert the drug to the active form. The genotype test would be used to find out who has these variants so they can be treated with an alternative drug.
A consultation has begun on the draft recommendations and consultees can have their say via nice.org.uk until Friday, 9 June 2023.

COVID-19
The Origins of Covid-19 — Why It Matters (and Why It Doesn't). Lawrence O Gostin, Gigi K Gronvall
https://www.nejm.org/doi/full/10.1056/NEJMp2305081?query=TOC&cid=NEJM%20eToc,%20June%2022,%202023%20DM2243420_NEJM_Non_Subscriber&bid=1628225867
When health emergencies arise, scientists seek to discover the cause — such as how a pathogen emerged and spread — because this knowledge can enhance our understanding of risks and strategies for prevention, preparedness, and mitigation. Yet well into the fourth year of the Covid-19 pandemic, intense political and scientific debates about its origins continue. The two major hypotheses are a natural zoonotic spillover, most likely occurring at the Huanan Seafood Wholesale Market, and a laboratory leak from the Wuhan Institute of Virology (WIV). It is worth examining the efforts to discover the origins of SARS-CoV-2, the political obstacles, and what the evidence tells us. This evidence can help clarify the virus's evolutionary path. But regardless of the origins of the virus, there are steps the global community can take to reduce future pandemic threats.
…After all the world has suffered in loss of life, economic hardship, and exacerbated health disparities, there is intrinsic value in knowing the cause. An objectively determined body of scientific facts cannot fully defuse the political rhetoric surrounding the origins investigation, but the search must continue.

Health Care in Its Original Position: Assessing the Ethical Response to the Covid-19 Pandemic
Jens Deerberg-Wittram, Thomas H Lee, NEJM Catalyst June 23, 2023
https://catalyst.nejm.org/doi/full/10.1056/CAT.23.0157
A half century after John Rawls published his philosophy masterpiece, A Theory of Justice, the authors ask if health care providers around the world responded to Covid-19 just as Rawls would have wanted — and whether applying Rawls' principles can help us address the challenges that lie ahead.

During the early phases of the Covid-19 pandemic, so little was known about the virus that many health care providers strove to function as Rawls might have wished — making decisions so equitably that mortality for admitted patients did not differ among subgroups defined by race at many institutions. (It is worth acknowledging that while initial treatment decisions were made without consideration of race in some locations, outcomes were still inequitable in many places because of systemic issues.)

...in the face of a global pandemic, professionals agreed on an integrated care model that in many ways was more equitable and efficient than established care systems.
Rawls J. A Theory of Justice. Reissue ed. Cambridge, MA: Belknap Press, 2005.
Google Scholar

Thaweethai T, Jolley SE, Karlson EW, et al. Development of a Definition of Postacute Sequelae of SARS-CoV-2 Infection. JAMA. 2023;329(22):1934–1946. doi:10.1001/jama.2023.8823
https://jamanetwork.com/journals/jama/fullarticle/2805540

Tala Ballouz, Dominik Menges, Alexia Anagnostopoulos, Anja Domenghino, Hélène E Aschmann, Anja Frei, Jan S Fehr, Milo A Puhan. Recovery and symptom trajectories up to two years after SARS-CoV-2 infection: population based, longitudinal cohort study. BMJ 2023;381:e074425 doi: https://doi.org/10.1136/bmj-2022-074425 (Published 31 May 2023)

Publications

Uncertainty Management in Regulatory and Health Technology Assessment Decision-Making on Drugs: Guidance of the HTAi-DIA Working Group.
Milou Hogervorst, Rick Vreman, Inkatuuli Heikkinen, Indranil Bagchi, Inaki Gutierrez-Ibarluzea, Bettina Ryll, Hans-Georg Eichler, Elena Petelos, Sean Tunis, Claudine Sapede, Wim Goettsch, Rosanne Janssens, Isabelle Huys, Liese Barbier, Deirdre DeJean, Valentina Strammiello, Dimitra Lingri, Melinda Goodall, Magdalini Papadaki, Massoud Toussi, Despina Voulgaraki, Ania Mitan, Wija Oortwijn.
International Journal of Technology Assessment in Health Care
doi: 10.1017/S0266462323000375, 25 pages. Published Online on 16 June 2023

Ivett Jakab, Maria Dimitrova, François Houÿez, Tamás Bereczky, Miroslava Fövényes, Zorana Maravic, Ivica Belina, Christian Andriciuc, Krisztina Tóth, Oresta Piniazhko, Rok Hren, Iñaki Gutiérrez-Ibarluzea, Marcin Czech, Tomas Tesar, Maciej Niewada, László Lorenzovici, Maria Stefanova Kamusheva, Manoela Manova, Alexandra Tzvetanova Savova, Zornitsa Emilova Mitkova, Konstantin Tihomirov Tachkov, Bertalan Németh, Zsuzsanna Ida Petykó, Dalia M Dawoud, Diana Delnoij, Saskia Knies, Wim Goettsch, Zoltán Kaló (2023). Recommendations for Patient Involvement in Health Technology Assessment in Central and Eastern European Countries. Front. Public Health 11:1176200. doi: 10.3389/fpubh.2023.1176200
http://journal.frontiersin.org/article/10.3389/fpubh.2023.1176200/full?&utm_source=Email_to_rerev_&utm_medium=Email&utm_content=T1_11.5e5_reviewer&utm_campaign=Email_publication&journalName=Frontiers_in_Public_Health&id=1176200

Expert opinion on a consensus-based checklist for the critical appraisal of cost-of-illness (COI) studies: qualitative interviews. Lena Schnitzler, Aggie TG. Paulus, Silvia MAA Evers, Tracy E Roberts, Louise J Jackson. International Journal of Technology Assessment in Health Care doi: 10.1017/S0266462323000181, 7 pages. Published Online on 9 June 2023

A consensus-based checklist for the critical appraisal of cost-of-illness (COI) studies. Lena Schnitzler, Tracy E Roberts, Louise J Jackson, Aggie TG Paulus, Silvia MAA Evers. International Journal of Technology Assessment in Health Care doi: 10.1017/S0266462323000193, 10 pages. Published Online on 16 June 2023

Healthcare priority-setting criteria and social values in Iran: an investigation of local evidence . Healthcare priority-setting criteria and social values in Iran: an investigation of local evidence Zahra Goudarzi, Leon Bijlmakers, Mojtaba Nouhi, Reza Jahangiri, Majid Heydari, Warren Simangolwa, Seyyed Mostafa Hakimzadeh, Karen Trujillo Jara. International Journal of Technology Assessment in Health Care doi: 10.1017/S0266462323000302, 9 pages. Published Online on 19 June 2023

Best practice considerations on the assessment of robotic-assisted surgical systems: results from an international consensus expert panel. J Erskine, P Abrishami, R Charter, A Cicchetti, R Culbertson, E Faria, J C Hiatt, J Khan, G Maddern, A Patel, K H Rha, P C Shah, P Sooriakumaran, S Tackett, G Turchetti, A Chalkidou. International Journal of Technology Assessment in Health Care doi: 10.1017/S0266462323000314, 28 pages. Published Online on 5 June 2023

Emerging healthcare interventions: Patient-Centered Outcomes Research Institute's programmatic initiative. Gowri Raman, Meghana Vijaysimha, Emma Kopleff, Kelly Dunham, Greg Martin, Jean Slutsky, William Lawrence. International Journal of Technology Assessment in Health Care doi: 10.1017/S0266462323000284, 8 pages. Published Online on 20 June 2023

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Industry funding of patient organisations in the UK: a retrospective study of commercial determinants, funding concentration and disease prevalence. Gentilini A, Parvanova I.
BMJOpen 2023;13:e071138. doi: 10.1136/bmjopen-2022-071138
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Patient organisations—not-for-profit organisations mainly composed of patients and/or caregivers that represent and support the needs of patients or caregivers—play an important role in the development, regulatory review and adoption of new drugs.
To assess the relationship between UK-based patient organisation funding and companies' commercial interests in rare and non-rare diseases in 2020. 1422 payments were made by 74 companies to 341 patient organisations. Almost all funds (90% of amounting to £22.6 million) from pharmaceutical companies were directed to patient organisations that are aligned with companies' approved drug portfolios and research and development pipelines. Despite rare diseases affecting less than 5% of the UK population, more than 20% of all payments were directed to patient organisations which target such conditions. Patient organisations focusing on rare diseases relied on payments from fewer companies compared to organisations focusing on non-rare diseases. Overall, diseases of the nervous system (£4.3 million) was the most funded therapeutic area over time, followed by neoplasms (£3.2 million) and endocrine, nutritional or metabolic diseases (£3.4 million). The conditions that received more funding in 2020 were multiple sclerosis (£1.7 million), followed by obesity (£1.4 million) and epilepsy (£1 million).
Increased independence of patient organisations could help avoid conflicts of interest. In the long-term, policymakers should make sure that patient organisations receive adequate public funding regardless of whether they focus on conditions that are profitable for the industry.

Challenges and Solutions for the Benefit Assessment of Tumor-Agnostic Therapies in Germany
Schiller, Juliane et al. Value in Health 2023;26(6):854-864
Precision medicine is increasingly important in cancer treatment. Tumor-agnostic therapies are used regardless of tumor entity because they target specific biomarkers in tumors. In Germany, the benefit assessment of oncological pharmaceuticals has traditionally been entity specific. Thus, the assessment of tumor-agnostic therapies leaves stakeholders with various challenges. To identify relevant challenges, we conducted qualitative interviews with different stakeholders who were involved in previous benefit assessments of tumor-agnostic therapies in Germany. To identify possible solutions for these challenges, we systematically searched the literature and the websites of European health technology assessment bodies. The absence of direct comparative studies, the use of basket studies and indirect comparisons, determining the appropriate comparative therapy, and system side challenges were identified. Seven

categories of solutions were identified, including an increased use of real-world evidence, making conditional decisions in the context of systematic reassessments, splitting the field of application, and finding (new) ways to design and analyse basket studies.

Financing and Reimbursement of Approved Advanced Therapies in Several European Countries
Iglesias-López, Carolina et al. Value in Health 2023;26(6):841-853
The uncertainty in the cost-benefit of advanced therapy medicinal products (ATMPs) is a current challenge for their reimbursement in health systems. This study aimed to provide a comparative analysis of the National Health Authorities (NHAs) reimbursement recommendations issued in different European countries. The reimbursement recommendations for the approved ATMPs were compared among 8 European Union (EU) Countries (EU8: Ireland, England/Wales, Scotland, The Netherlands, France, Germany, Spain, and Italy). A total of 19 approved ATMPs and 76 appraisal reports were analyzed. The majority of the ATMPs were reimbursed, although with uncertainty in added therapeutic value. No relationship between the type of the European Medicines Agency approval and reimbursement was found. Managed entry agreements, such as payment by results, were necessary to ensure market access. The main issue during the evaluation was to base the cost-effectiveness analyses on assumptions because of the limited long-term data. The estimated incremental cost-effectiveness ratio among countries reveals high variability. Overall, the median time to NHA recommendation for the EU8 is in the range of 9 to 17 months.

Measuring Financial Toxicity (FT): A Closer Look. MR LeBlanc, et al. Value in Health 2023;26(6):953-955
In 2013, the term FT was introduced into the health sciences literature to describe the patient-level impacts of the cost of cancer care. Inconsistent terms and definitions have hindered research. New nomenclature and conceptualization spurred the research community to investigate this important aspect of patient experience in a more harmonized approach. As we enter the second decade of FT research, a look back on what we have learned so far is warranted.

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