

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

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 from the Chair

Dear PCiG members,

We have had a very successful conference in Rome. It was truly wonderful to see many of you there and to spend some quality time together over dinner. I was struck by how many sessions involved the concepts of patient and citizen involvement, and it certainly felt as though our work was central to many of the debates on how HTA is evolving. We kicked off the conference with our pre-conference workshop which had very high attendance and generated some valuable debate. As always, we will take the outputs from this workshop to help shape our work going forward. Similarly, at our annual business meeting, the members attending were brimming over with ideas on how we could align the work of this group with some of the activities that are happening across HTAi and amongst other bodies. This has spurred some really important thinking on how we prioritise our work and focus on the areas that we need to.

In the rest of the E-Bulletin, you will see some of the summaries from just a few of the sessions in Rome, there were many more. If any of you presented at Rome and would like to share your slides with the membership, then please do. It will help those that were not able to attend the conference to get a flavour of the diversity and depth of thinking that is taking shape around patient and citizen involvement.

I wish you all a good month with enough sun to keep you happy (no matter which hemisphere you live in!).

Neil Bertelsen

Report on the PCIG workshop and panel session:

Workshop highlights need to increase benefit and reduce burden of patient involvement

The need for better collaboration among HTA bodies, researchers and patient groups to increase the benefit and reduce the burden of patient involvement in HTA was highlighted at a PCIG workshop at the HTAi Annual Meeting in Rome. More than 80 people attended the full-day workshop on 18 June which included presentations by 30 authors and contributors to the recently released book *Patient Involvement in Health Technology Assessment* (available at: <http://www.springer.com/gb/book/9789811040672>). The day provided an overview of how patient involvement can be conceptualised, methods for research into patient aspects, approaches to participation and evaluation. It also included panel sessions with representatives from HTA bodies providing examples of how patient-based evidence and patient input has made a

difference to HTAs. Discussions throughout the day focused on the need to share knowledge, skills and data and develop HTA processes which enable patients and patient groups to efficiently and effectively contribute to assessments.

PowerPoint presentations from the day will be published on the interest group website. Additionally, some presentations are being written up for publication and PCIG is using feedback from the day to plan its work programme, including prioritising topics for educational webinars on methodology and approaches.

Panel Session Title: Patient Involvement: Unique needs of developing countries

Jointly hosted by the Developing Countries Interest Group and the Patient and Citizen Involvement Interest Group, the needs of developing HTA systems were explored across four case studies to highlight the areas of support needed to embed patient involvement in HTA processes and to define the unique needs within developing HTA systems compared to more established bodies. Antonio Ciaglia from IAPO started the session by bringing a global perspective to the needs of developing and emerging HTA systems. He explained that solid democratic systems and accountability go hand-in-hand with the development of transparent HTA processes that take account of the patient and citizen perspective. Next, Li-Ying Huang (Grace), from Taiwan, explained the evolving nature of patient involvement in the Taiwanese HTA system. She explained that the system of using an on-line consultation process is being supported by the work of TAPO, the Taiwan Alliance of Patient Organisations. This organization was only founded in 2016, but already has an ambitious plan to improve education for patient organisations on HTA issues in Taiwan. Aline Silveira Silva, from Brazil's CONITEC, gave the next perspective, showing how Brazil is heavily engaged in large public consultation processes. She explained that on top of these consultations, Brazil is bringing participation of patient representatives into the plenary session discussion for certain treatments and that this approach could be a big opportunity for the future. Joice Valentim, representing the HTAi Interest Group on Developing Countries, discussed the general situation in Latin America. Thinking in the region has been boosted by the HTAi Latin America Policy Forum, and a focus on patient involvement is expected over the coming years. Finally, Stanimir Hasardzhiev, chairman of the National Patient Organisation in Bulgaria and board member of the European Patients' Forum discussed the situation in Bulgaria and a very exciting initiative across the region. He explained that a standard procedure for involving patients in the HTA is being developed for Bulgaria, but that HTA is still perceived as a delaying tactic, rather than a true assessment for timely reimbursement decisions. A think tank has been set up across Central and Eastern European countries to look at the issues. This has not been set up by the government, but is formed of patient representatives, healthcare professionals and HTA and pharmacoeconomic experts to see if it possible to propose new ways of working that will improve access across the region. This think tank has been formed to look at the key challenges across the region and to deliver a pilot project on regional joint HTA for Bulgaria, Romania, Hungary, Croatia and Slovakia using a pragmatic value assessment model.

Neil Bertelsen

HTAi Annual Meeting 2017: Explore how the global HTA community is evolving towards an integrated ecosystem – from local needs to global opportunities

June 17-21, 2017 at the Ergife Palace Hotel in Rome, Italy. Biggest ever HTAi meeting, with nearly 1200 people from over 70 countries

Daniel Lalonde, the new Executive Director for the HTAi Secretariat was in attendance, as was Dr Yingyao Chen who chairs the Interest Group Steering Committee. Daniel has taken over from Lucy Turner.

Julia Abelson and McMaster team awarded Egon Jonsson Award

A team of health policy experts led by McMaster University's Julia Abelson have been awarded the 2017 Egon Jonsson Award in recognition of excellence in contributions made to the International Journal of Technology Assessment in Health Care. Abelson is an adjunct faculty member of the McMaster Health Forum, as well as a Professor in McMaster's Department of Health Evidence and Impact and a member of the Centre of Health Economics and Policy Analysis. The award also recognized co-authors John Lavis and

François-Pierre Gauvin of the McMaster Health Forum, as well as researchers and collaborators from other institutions across Ontario.

The Egon Jonsson Award is given annually by Health Technology Assessment international (HTAi) to the authors of an outstanding paper in the society's official journal published over the past year. The Award is named in recognition of Professor Egon Jonsson, Editor-in-Chief of the International Journal of Technology Assessment in Health Care from its founding in 1985 until 2010. The 2017 award was recently presented at HTAi's conference in Rome.

<https://www.mcmasterhealthforum.org/new-at-the-forum/2017/06/21/mcmaster-health-policy-experts-awarded-2017-egon-jonsson-award>

The paper: Abelson J, Wagner F, DeJean D, Boesveld S, Gauvin FP, Bean S, Axler R, Petersen S, Baidoobonso S, Pron G, Giacomini M, Lavis J. Public and patient involvement in health technology Assessment: A framework for action. *A. Int J Technol Assess Health Care*. 2016 Jan;32(4):256-264. Epub 2016 Sep 27.

HTAi Patient Group submission templates for HTA of diagnostic technologies

At the Annual Meeting, we launched the HTAi Patient Group submission templates for HTA of diagnostic technologies. Thanks go to Laura Norburn and Lizzie Thomas of NICE and Victoria Wurcel who led the development of this template. See all the documents in the patient group submissions section of this web page.

<http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-hta-agencies-and-policy-makers.html>

Karen Facey

About the Rome Annual Meeting from members:

It was an honor to share those days with many of you in Rome. Thank you for the workshop and panel organization, mainly to Karen and Neil, for the usual kindness and patience. We learned a lot with other countries experiences.

In some talks with colleagues from Latin America, we found that is necessary for us to be closer, for exchange our experience. And we've already started doing this.

I came home very excited for innovate and to do more about patient engagement. So, I am looking for some internship opportunity (between 4 - 8 weeks) about patient involvement or qualitative research with agencies that involve patient and public strongly, preferable in some English speaking country. Please, let me know about some opportunity.

Aline Silveira Silva, Executive Secretariat of the National Committee for Health Technology Incorporation – CONITEC, Brazilian Ministry of Health

I would like to introduce myself as I only recently joined the Patient and Citizen Involvement Interest Group. My name is Elisabeth Oehrlein and I am a PhD Candidate at the University of Maryland, Baltimore in the Dept. of Pharmaceutical Health Services Research. My research is on the Patient-Focused Drug Development, patient engagement in value assessment, and stakeholder engagement to improve study designs for secondary claims analyses. I really enjoyed attending the PCIG's open meeting, dinner, and research sessions and look forward to becoming involved in PCIG initiatives over the coming year. I am also looking into purchasing a copy of "Patient Involvement in Health Technology Assessment" for my department.

Elisabeth M Oehrlein, PhD Candidate, University of Maryland School of Pharmacy

The HTAi conference was exciting for those of us focused on patient engagement activities; the workshop on 'Patient Involvement in HTA' got the week off to a great start and by end of the week, I felt that patient

engagement/involvement had finally gone mainstream! Here are two of the highlights I took away from Rome:

- “Patient fatigue” concerning input to HTA was something I felt was an important call-out, that we need to do things differently, e.g. have frameworks in place and do things more internationally and collaboratively, not that it is treated as a template-filling exercise. In particular, frustration was apparent when patients haven’t been involved early on to understand their needs, nor in the clinical study design or interpretation, and hence the evidence submitted to HTA bodies is not addressing those things important to them; yet they are still expected to speak up or input to the technology assessment. “If the data isn’t right, the patient voice doesn’t help, so let’s get it right upstream”.
This was an important call-to-action for industry, to do a better job of engaging with patients earlier in designing trials that address their needs. That will also increase the likelihood and frequency of the patient voice influencing HTA-decision-making.
- Impact of patient input: Heidi Livingstone (NICE) gave a great example in ankylosing spondylitis, where the (comparatively small) patient support group used both 3 years’ of member survey data, plus a bespoke survey of >600 patients (done in 4 weeks) as part of their submission during a technology appraisal. Subsequently they did a follow-up survey in 8 days to address questions raised by the appraisal committee regarding gaps in their understanding (e.g. ‘if you don’t respond to one anti-TNF, will you respond to a different one?’). Deb Maskens similarly described how Kidney Cancer Canada are using their registry/network and linking this to EMR data, to address important clinical questions and provide as input to technology appraisals. *I felt both examples raise the patient voice and evidence input to entirely new levels!*

Nigel Cook, Novartis Pharma AG Switzerland

EPF participated in the latest HTAi Conference in Rome, where international experts discussed the way forward for HTA processes, including a more holistic and multi-criteria approach, with increased patient involvement

... “a globalised HTA can be a supporting mechanism for countries with scarce resources” (Stefano Vella, President of AIFA (Italian Medicines Agency)).

Patient involvement in HTA was another important topic of the conference, from its conceptualisation, through the definition of methodologies to increase its level, to its evaluation. New methodologies have been developed to gather patient input, such as for instance social media analytical methods, as outlined in the recent book to which EPF contributed.

The closing session focused on the ethical dilemmas of HTA and the need for decision-makers to have access to relevant data to make informed-decisions and mitigate the tension between public health and specific needs of patients living with chronic conditions.

Guido Rasi, Executive Director of the European Medicines Agency (EMA) explained that using common sets of data and methodologies in evidence generation for HTA decisions would generate efficiency gains for the entire process. He also stressed that “*pricing and reimbursement decisions should never compromise or challenge regulatory decisions*”.

Concluding the day, Prof Norman Daniels stressed that current HTA standard tools “*leave important questions unanswered*” and presented his theory of “*Accountability for Reasonableness*”. According to him, embedding HTA in a fair deliberative process, would help answer the questions decision-makers ought to address on the impact of their actions. He then listed the sine-qua-non conditions to be met in order to make a well-informed decision:

- Publicity/transparency of the process;
- Revising the decisions in case of new available evidence;
- Enforceability of the decisions made.

From a patient perspective, the fairness of the process and inclusion of these conditions are key to the acceptability of decisions and should alleviate the sense of competition that sometimes can be found within the patient community.

Valentina Strammiello, European Patients Forum

<http://www.eu-patient.eu/News/News/htai-conference-towards-a-globalised-approach-and-patient-involvement/>

The following presentations from Rome have been received:

They are being put on our webpages so that they can be readily accessed. We are happy to add to this number, so you can still send yours in!

Patient Involvement in HTA: unique needs of developing countries. Case-study - Brazil

Aline Silveira Silva, National Committee for Health Technology Incorporation – CONITEC

Poster. Patient and Public Involvement in HTA. The Brazilian Experience

Aline Silveira Silva

INAHTA Panel. Patient involvement in HTA: Experiences from the front line of HTA production

Panel Session, Moderator Irina Cleemput, Belgium. Panellists Ken Bond, Canada; Sophie Werko, Sweden; Grace Li Ying Huang, Chinese Taipei; Carlos Pinzón; and:
Patient involvement in HAS HTA by Hervé Nabarette HTA Division, Technical advisor

Poster. Monitoring the HAS' Initiative on Patient Contribution to Rapid HTA

Hervé Nabarette, Chantal Bêlorgey, French National Authority for Health (HAS)

Patients' preferences and engaging patients at NICE

Lizzie Thomas, Public Involvement Programme, NICE

Patients' views of HTA at NICE: Enhancing Involvement Opportunities

Lizzie Thomas, Public Involvement Programme, NICE

NICE Technology Appraisal Patient Expert Feedback: 15 Month Analysis

Chloe Kastoryano, Public Involvement Programme, NICE

Patients' views on providing evidence; feeding into the HTA ecosystem

Heidi Livingstone, Public Involvement Programme, NICE

Survey of HTA Evaluation Strategies for Patient And Public Involvement under leadership of Laura Weeks, which I presented in Rome. Anke-Peggy Holtorf, PhD, MBA

Are journal editors a barrier to publication of real world evidence?

Elisabeth Oehrlein, Jennifer S. Graff, PharmD, Eleanor PerfeCo, PhD, MS, C. Daniel Mullins, PhD, Robert W. Dubois, MD, PhD, Chinenye Anyanwu, PharmD, MPH, Ebere Onukwugha, MS, PhD

Landscape Assessment: Patient Engagement in Health Technology Assessment

Elisabeth Oehrlein, BA, Jason Harris, Nicole Labkoff, Eleanor Perfetto, PhD, MS, Marc Boutin, JD

Improving the Patient Centricity of Value Assessments: A Rubric

Eleanor M. Perfetto, PhD, MS, Elisabeth Oehrlein, Marc Boutin, JD, Sarah Reid, and Eric Gascho

Vignette. The Use Of Ethnographic Fieldwork In HTA

Helle Ploug Hansen, Department of Public Health, University of Southern Denmark, hphansen@health.sdu.dk; Tine Tjørnhøj-Thomsen, University of Southern Denmark

Vignette. Identification of needs of Pigmented Villonodular Synovitis (PVNS) patients using online bulletin board (OBB)

Nigel Cook, Kyle Landskroner, Susann Walda, Olivia Weiss, and Vikrant Pallapotu
¹Novartis Pharma AG, Basel, Switzerland

The Importance of Patient Organisations Involvement in HTA

Kathi Apostolidis, Francesco De Lorenzo, Francesco Florindi, Lydia Makaroff
European Cancer Patient Coalition, Brussels, Belgium

Poster. The role of cancer patients in HTA & disinvestment: Recommendations from ECPC and CanCon *Value of Innovation in Oncology*

Prof Francesco de Lorenzo, Kathi Apostolidis

PCIG resources

The HTAi Patient & Citizen Involvement in Health Technology Assessment Interest Group sets up as Linked In group

<https://www.linkedin.com/company-beta/11168195>

Neil McGregor-Paterson

Patient Involvement in HTA – the book

The book, edited by Karen Facey, Helle Ploug Hansen and Anne Single, contains detailed explanations of methods for conducting patient aspects research, approaches to participation and 15 international case studies. *Patient Involvement in HTA* is aimed at researchers, postgraduate students, HTA professionals and experts in the HTA community.

Patient involvement in HTA is available in hardcopy or ebook from Springer:

<http://www.springer.com/gb/book/9789811040672> and Amazon: https://www.amazon.com/Patient-Involvement-Health-Technology-Assessment/dp/9811040672/ref=sr_1_fkmr1_1?s=books&ie=UTF8&qid=1495583998&sr=1-1-fkmr1&keywords=patient+involvement+in+HTA

What's happening

NICE to work with partners on developing new ways to measure quality of life across health and social care, 13 June 2017

<https://www.nice.org.uk/news/article/nice-to-work-with-partners-on-developing-new-ways-to-measure-quality-of-life-across-health-and-social-care>

BLUEPRINT FOR TRANSPARENCY AT THE U.S. FOOD AND DRUG ADMINISTRATION
RECOMMENDATIONS TO ADVANCE THE DEVELOPMENT OF SAFE AND EFFECTIVE MEDICAL PRODUCTS

http://www.jhsph.edu/departments/health-policy-and-management/_pdf/FDA_Transparency.pdf

Publications

Advancing the science of patient input throughout the regulatory decision-making process by MA Tegenge, MM Moncur, R Sokolic, RA Forshee, T Irony. Learning Health Systems 2017. Experience Report, accepted May 2017

The US Food and Drug Administration (FDA) understands the value of patient input in the regulatory decision-making process and has worked to enhance meaningful engagement. In recent years, there has been an increased scientific demand for more systematic and quantitative approaches to incorporate patient input throughout the medical product lifecycle, including to inform regulatory benefit-risk assessments. The use of patient preference information (PPI), elicited using established scientific methods, is a promising strategy for accomplishing this...

A learning healthcare system paradigm can also help us better understand and continuously improve the incorporation of the patient perspective in regulatory decision making. In this article, we highlight the Food and Drug Administration's Center for Biologics Evaluation and Research experience and current initiatives on advancing the science of patient input in a regulatory setting, in particular, PPI. We provide a use case that explores how the principles and benefits of PPI applied in shared clinical decision making can be realized and leveraged to enhance regulatory evaluation of innovative therapies....we compiled a list of example resources that support stakeholders in designing and conducting PPI studies.

Submitted by Nigel Cook

Survey Shows Substantial Impact of Rare Diseases on Daily Life, 28 June 2017

EURORDIS-Rare Diseases Europe ran the first Europe-wide survey on the social impact of rare diseases. Results demonstrate a serious impact on everyday life for over 80% of patients and families.

Over 3,000 respondents (including patients and carers) across Europe took the survey '*Juggling care and daily life: The balancing act of the rare disease community*'

The results show that the **time burden** of daily care management and care coordination for rare disease patients and families is substantial:

- **42%** of total respondents spend **more than 2 hours** a day on care for their disease.
- **62% of carers** reported they spend more than 2 hours per day on tasks related to the disease while **nearly a third spend more than 6 hours a day on care for a patient** (in comparison, OECD figures show that 50% of carers (for patients with any type of disease or dependent conditions) provide less than 1.4 hours of care a day).
- **38%** of respondents declare that they were absent from work due to health-related problems for over **30 days in the last 12 months**.
- **41%** of patients and carers responded they need special leave at work but could not obtain it.

[See the full results](#) or read the [press release](#).

<http://www.eu-patient.org/blog/?p=723>

From the EPF Newsletter

Blogs and things

The use of a web-based system that allowed patients to report symptoms to their clinicians remotely associated with benefits including improved quality of life and longer overall survival, 4 June 2017.

<http://am.asco.org/online-tool-reporting-symptoms-extends-survival>

Is the staggeringly profitable business of scientific publishing bad for science? 27 June 2017

Stephen Buranyi, The Guardian, long read

https://www.theguardian.com/science/2017/jun/27/profitable-business-scientific-publishing-bad-for-science?CMP=share_btn_tw



Saturated Biases: Where the AHA...posted on June 28, 2017 by Hilda Bastian

<http://blogs.plos.org/absolutely-maybe/2017/06/28/saturated-biases-where-the-aha-advice-on-coconut-oil-went-wrong/>

Webinars, tools and courses

Upcoming EUPATI webinar: Guidance on Patient Involvement in HTA 5 July 2017 - 17:00 to 18:30 CET

Moderator: Ingrid Klingmann, *European Forum for Good Clinical Practice*

To register:

<https://register.gotowebinar.com/register/3881915245336823554>

Guidance for Patient Involvement in HTA document:

<https://www.eupati.eu/health-technology-assessment/guidance-for-patient-involvement-in-hta/>

IMI GetReal online course 'Real-World Evidence in Medicine Development' – 2 Oct to 12 Nov 2017

<http://www.imi-getreal.eu/>

IMI GetReal has launched PragMagic - an innovative tool to assist pragmatic trial design

<http://www.imi-getreal.eu/Webinars/PragMagic-launch>

Janet Wale, HTAi PCIG

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