NEWS

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

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

*Enhanced quality and relevance of HTA through patient and citizen involvement*

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

- Welcome from the Chair
- HTAi2018 call for abstracts
- News from HTA agencies
- What's happening – news, blogs, publications

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**Welcome to this month's E-Bulletin**

Dear members,

It seems like only a moment ago that many of us were spending time together in Rome, sharing ideas and experiences. It may seem a long way off, but the next HTAi congress in Vancouver is already being prepared. Workshop and panel submissions are expected earlier than in past years, with a deadline of 16th October. So if you have not started planning your submissions, I urge you to do so now. The theme for the conference is "Strengthening the Evidence-to-Action Connection". This will include three plenary sessions that all support this theme; how HTA needs to change its approach to generating, synthesizing and presenting evidence in order to remain relevant in a rapidly changing world; what is required to enable the implementation of evidence into evidence-informed policies and practices, and what can be done to strengthen and accelerate the evidence-to-action connection.

We were all delighted at the number and quality of sessions in Rome from the members of this interest group, and we are sure that you will be full of ideas on what you can do next year. Below are the links for the abstract guidelines and the submission page.
Submit Abstract HTAi Submission Guidelines

We can't wait to see what you develop for Vancouver!
Neil Bertelsen

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## Welcome to the 2018 Annual Meeting Website, Vancouver, Canada, June, 2018

http://www.htai2018.org/call-for-abstracts-now-open/

Abstract submissions play a vital part of the scientific success of our upcoming HTAi 2018 Annual Meeting. Submission deadlines are different based on submission type. Please note below important dates regarding abstract submissions. No extensions to these deadlines will be made.

Deadline for Workshop & Panel submissions: **October 16, 2017**
Deadline for Oral, Vignette & Poster Presentation submissions: **November 24, 2017**

Please review the Abstract Submission Guidelines before submitting an abstract.
Receipt of abstract submission will be acknowledged via e-mail prior to submission close for each category.

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**HTAi celebrates its 15th Annual Meeting in Vancouver from June 1-5, 2018**, providing an unparalleled opportunity to consider, debate and clarify the role of health technology assessment (HTA) in the health care ecosystem. Revolving around the Annual Meeting's central theme "Strengthening the Evidence-to-Action Connection", HTAi 2018 welcomes all stakeholders – patients/consumers, health care providers, academic researchers, HTA agencies, payers, policy makers, industry – bringing together more than 1000 delegates from around the globe to discuss the latest advances in this constantly evolving field.
HTAi2018.org #HTAiVancouver2018
Promotional information prepared for your use. HTAi2018.org/Media:

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**Did you complete the PCIG questionnaire by 30 September 2017? If not, it is not too late…**
Does your organisation have or access resources for patients and citizens to be able to contribute to HTA processes?
http://www.htai.org/interest-groups/patient-and-citizen-involvement/pcig-home/questionnaire.html
Please complete our questionnaire to provide us with your web links – so that we can collate a useful widely accessible resource. Send your completed questionnaire (along with any extra resources in the email) to: **htai.pie@gmail.com**

*Members of the HTAi Patient and Citizen Involvement in HTA Interest Group, Patient Involvement and Education Working Group (PIE)*

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**HTA Agency News**

**HAS on patient perspective**
HAS finished its test phase concerning a new procedure to include the patient perspective in rapid HTA on drugs and devices. This new procedure, which consists of written submissions from patient organizations (POs), is considered a success. During the first 6 months, 18 POs participated sending 24 contributions, concerning 22 drugs.
The HAS Board decided to continue this procedure and to adapt it: increasing the time for POs to contribute (45 days in place of 30 days), publishing the contributions on the HAS website, improvement of the presentation of the submission to the committee.
The new rules for PO contributions have been posted on HAS website the 25th of September.
https://www.has-sante.fr/portail/jcms/c_2666630/fr/contribution-des-associations-de-patients-et-d-usagers-aux-evaluations-de-medicaments-et-dispositifs-medicaux

Hervé Nabarette

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**Canada – CADTH**
In October, CADTH is hosting a Drug Portfolio Information Sessions for patient groups, clinicians, pharmaceutical companies, and other interested stakeholders. The session will cover international trends in HTA, patient engagement, and will describe CADTH's Health Technology Management Strategy. More on CADTH's strategy can be found at https://www.cadth.ca/better-health-better-patient-experience-better-value-transforming-how-we-manage-health-technologies. CADTH will also be hosting an in-person meeting of the CADTH Patient Community Liaison Forum to build a shared understanding of health technology management and identify corresponding opportunities for patient involvement to strengthen patient involvement at CADTH.

**CADTH Symposium 2018**
At CADTH, the call has gone out for abstracts to the 2018 symposium, in Halifax, Nova Scotia. The symposium's theme is Managing Health Technologies: Supporting Appropriate, Affordable, and Accessible Care. Once again, CADTH invites those working in a not-for-profit, patient-related organization, or a

citizen's organization interested in health policy, to apply for a travel award to support attendance. More details can be found at: https://www.cadth.ca/2018-cadth-symposium

Sarah Berglas

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**What's Happening**

**Access to online communities as a powerful measurement tool:**
National Quality Forum and PatientsLikeMe joint project using online communities. **Measuring What Matters to Patients: Innovations in Integrating the Patient Experience into Development of Meaningful Performance Measures**. Date of Publication: August 2017
https://www.qualityforum.org/Publications/2017/08/Measuring_What_Matters_to_Patients__Innovations_in_Integrating_the_Patient_Experience_into_Development_of_Meaningful_Performance_Measures.aspx

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**EURORDIS Rare Barometer Programme**
http://www.eurordis.org/rare-barometer-programme
At EURORDIS, requests for patient perspectives in health, research and social policy-making are on the rise as the benefits associated with evidence-based programmes or policies are being increasingly recognised and required by all stakeholders. To best respond to our growing advocacy role and achieve a high quality evidence-base from people living with a rare disease, the EURORDIS Rare Barometer Programme will rely heavily on patient engagement to inform sound policy from the patient perspective by carrying out surveys, focus groups, individual face-to-face interviews and other opinion-gathering methods to gain firsthand feedback from patients.
Rare Barometer Voices is a community of people living with a rare disease who are willing to participate in EURORDIS-Rare Diseases Europe surveys and studies. Read about who can participate and how to register at http://www.eurordis.org/voices

Submitted by Marleen Kaatee

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**EURORDIS Summer School 2018: Barcelona 11-15 June 2018**
http://www.eurordis.org/news/apply-eurordis-summer-school-2018
2018 marks the tenth anniversary of the Expert Patient and Researcher EURORDIS Summer School! 50 participants will be selected to take part in the training held in Barcelona from 11 – 15 June. If you would like to be considered to take part in the 2018 edition, find out more about the application process (opening 16 October).
The EURORDIS Summer School on medicines research and development is part of the wider EURORDIS Training Programme, which enables patients and researchers to gain an understanding of the regulatory process for the development of rare disease medicines.
Since 2015, the Summer School has welcomed both researchers as well as patients, creating an opportunity for exchange and discussion between the two groups. The Summer School is made up of a pre-training module and then the on-site training for one week during June.
In addition, EURORDIS has free online modules available to all, which focus on the same topics covered during the Summer School including: clinical trials methodology, clinical research, ethical considerations in medicines development, regulatory affairs and health technology assessment.

Submitted by Karen Facey

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**Publications**

**Patient value: Perspectives from the advocacy community**

Authors: Bonnie J Addario, Ana Fadich, Jesme Fox, Linda Krebs, Deborah Maskens, Kathy Oliver, Erin Schwartz, Gilliosa Spurrier-Bernard, Timothy Turnham. Health Expect. 2017;00:1-7. Open access review article
http://onlinelibrary.wiley.com/doi/10.1111/hex.12628/full/
All health-care systems are under financial pressure and many have therefore developed value frameworks to assist decision making regarding access to treatment. Unfortunately, many frameworks simply reflect the clinically focused values held by health-care professionals rather than outcomes that also matter to patients. It is difficult to define one single homogeneous set of patient values as these are shaped by social, religious and cultural factors, and health-care environment, as well as many factors such as age, gender, education, family and friends and personal finances. Instead of focusing on an aggregated set of values, frameworks should attempt to incorporate the broader range of outcomes that patients may regard as more relevant. Patient advocates are well placed to advise assessment bodies on how particular therapies will impact the patient population under consideration and should be closely involved in developing value frameworks. In this paper, a group of patient advocates explore the varying definitions of patient value and make positive recommendations for working together to strengthen the patient voice in this area.

Submitted by Deb Maskens

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**Practical Implications of Using Real-World Evidence (RWE) in Comparative Effectiveness Research: Learnings from IMI-GetReal**
By: Amr Makady, Heather Stegenga, Antonio Ciaglia, Thomas PA Debray, Michael Lees, Michael Happich, Bettina Ryll, Keith Abrams, Rob Thwaites, Sarah Garner, Páll Jonsson, Wim Goettsch & on behalf of GetReal Work Packages 1 & 4. J. Comp. Eff. Res. (Epub ahead of print)
The current state of accessibility to RWE experienced during IMI-GetReal case studies and stakeholders beyond the consortium poses a considerable barrier to furthering RWE use in comparative effectiveness research (CER) and healthcare decision-making. Bearing in mind that such data is generated by patients in clinical practice, this barrier diminishes the potential benefit of using RWE to provide critical insights on the effectiveness of treatments for all patients in real practice; insights that randomized controlled clinical trials (RCTs) are often not designed to provide. An array of potential solutions lend themselves to overcoming this persistent inaccessibility to RWE and maximizing societal gain from its use in CER. However, the choice regarding which path to take, addressing trade-offs associated with such a choice, as well as its implementation, requires a collaborative effort spanning all relevant stakeholders; from decision-makers, to industry and patient representatives.
A major drawback of aggregate data (AD) is the limited ability to explore individual patient characteristics which may influence or confound treatment outcomes. Therefore, in order to conduct robust CER that can inform decision-making, HTA agencies and payers often require more sophisticated analyses to be conducted whereby researchers can adjust for individual patient characteristics to generate more accurate estimates of effectiveness. Importantly, such strategies require analyses based on individual patient-level data (IPD).
IMI-GetReal. www.imi-getreal.eu/

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**The Impact of Participation in Online Cancer Communities on Patient Reported Outcomes: Systematic Review**
By: Mies C van Eenbergen, Lonneke V van de Poll-Franse, Peter Heine, Floortje Mols
JMIR Cancer 2017 (Sep 28); 3(2):e15
HTML (open access): http://cancer.jmir.org/2017/2/e15
PDF (free): http://cancer.jmir.org/2017/2/e15/PDF
In recent years, the question of how patients' participating in online communities affects various patient reported outcomes (PROs) has been investigated in several ways.
This study aimed to systematically review all relevant literature identified using key search terms, with regard to, first, changes in PROs for cancer patients who participate in online communities and, second, the characteristics of patients who report such effects....

**Patient Experience Data May Require Separate Label, Genentech Suggests.** The Pink Sheet, September 26, 2017

The increasingly patient-centric approach to drug development and regulation may necessitate the development of separate product labeling for patients, one industry researcher believes. Speaking at a Sept. 18 workshop on the US FDA's benefit/risk assessment framework, Alicyn Campbell, global head of Patient-Centered Outcomes Research for Oncology Product Development at Genentech Inc., questioned whether the time has come for separate, patient friendly labeling to reflect the growing body of patient-reported data on drug side effects, efficacy and overall experience in a way that is understandable to those likely to use the treatment…

Systematic inclusion of the patient voice in clinical trials creates large amounts of data that frequently requires descriptive analysis and presentation at the item/concept level "because if I'm a patient who really cares about those side effects of interest, we do need to get into that level of detail," she said.…

The example given is Rituxan Hycela (rituximab/hyaluronidase), a subcutaneous form of the CD20-directed cytolytic antibody that previously was approved only for intravenous infusion… The Clinical Trials section of Hycela labeling includes a subhead for "Patient Experience" that reflects data from a dedicated patient preference study in previously untreated adult patients outside the US. The study evaluated patient preference and satisfaction for the subcutaneous route of administration compared to intravenous infusion. The data were included in Genentech's briefing document for an FDA advisory committee review of the new formulation…

In the patient preference study, 77% of subjects favored subcutaneous administration with Rituxan Hycela over intravenous rituximab, with the most common reason being less time in the clinic.…Campbell presented more data from the patient preference study in the form of a bar chart showing more reasons why patients preferred subcutaneous rituximab over intravenous. The top-line preference data were included in the Hycela labeling "in a verbal, descriptive format rather than in a bar chart format. …

**How Patient Preferences Contribute to Regulatory Decisions for Medical Devices**. 25 September 2017
By: Jeffrey Shuren, Anindita Saha and Martin Ho.
https://blogs.fda.gov/fdavoice/index.php/2017/09/how-patient-preferences-contribute-to-regulatory-decisions-for-medical-devices/

**Jeffrey Shuren**, Director of FDA's Center for Devices and Radiological Health: Since we launched our Patient Preference Initiative as part of our medical device regulatory decision-making process in September 2013, we've seen increasing evidence of the benefits of soliciting patient feedback – most recently, in giving kidney patients more therapy options and enhancing the safe use of a glucose monitor by pediatric patients with Type 1 diabetes. In August, for the first time we cleared an expanded indication for a home hemodialysis machine so it could be used without a care partner being present, a decision based in part on asking kidney patients about their tolerance for risk…

**Anindita Saha**: During our August 2015 public workshop as part of the Kidney Health Initiative, a public-private partnership, multiple patient representatives argued that the care partner requirement effectively ruled out home treatment for those patients who lived alone or who could not afford to hire a care partner. We told them that we were willing to reconsider this issue but needed a systematic way to evaluate risk. A medical device developer, NxStage, approached us at the meeting to propose a patient-centric approach. During the pre-submission process, we worked with NxStage to design their robust patient survey that could quantify the level of risk that patients would accept in exchange for doing hemodialysis in the home alone instead of at a dialysis center. The survey used a weighting method described in the Patient Preference Framework developed by the Medical Device Innovation Consortium (MDIC), a nonprofit that operates in partnership with FDA to improve the medical technology environment.

**Martin Ho**: The System One is the latest example of how we have been successfully implementing our Patient Preference Initiative, designed to identify and develop methods for assessing patient valuations of benefit and risk related to specific device types and specific illnesses and conditions that can be used to inform product review decisions. Collecting qualitative feedback from patients is another important

technique and proved helpful in enhancing the safety of the Dexcom G5 Continuous Glucose Monitoring (CGM) System and Animas Vibe System, a continuous glucose monitor with an insulin pump, for children. FDA discussed with patients, care partners, and patient groups their concerns about the safety of using an insulin pump in the young pediatric population. These conversations included how the device would be used once approved by FDA for the young pediatric population and what kind of safety considerations might be relevant. Based on this feedback, FDA worked with the company to develop additional risk mitigation strategies that included a lockout feature to prevent unintended boluses by young children playing with the insulin pump. Patient preference information led to a safer device on the market, and parents can now have greater confidence with managing their children's diabetes.
We invite companies to start a conversation with FDA about using patient preference information to support your submission. For more information, you might want to attend our upcoming patient preference initiative meeting on December 7-8, 2017, where some of these topics will be further discussed… FDA encourages further research in this field.

Submitted by Nigel Cook

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**The fate of FDA postapproval studies**
By: Steven Woloshin, Lisa M. Schwartz, Brian White, and Thomas J. Moore. NEJM 2017 pp1114-7
The slow, irregular pace of postapproval studies contrasts starkly with the short, rigid deadlines and other shortcuts used to speed marketing approval. Only half the studies established in 2009 and 2010 had been completed by the end of 2015, and some companies even failed to submit required annual status reports.

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**R&D development costs of new drugs**
**Research and Development Spending to Bring a Single Cancer Drug to Market and Revenues After Approval**
By: Vinay Prasad, Sham Mailankody. JAMA Intern Med. Published online September 11, 2017.
http://jamanetwork.com/journals/jamainternalmedicine/article-abstract/2653012
In this analysis of US Securities and Exchange Commission filings for 10 cancer drugs, the median cost of developing a single cancer drug was $648.0 million. The median revenue after approval for such a drug was $1658.4 million.

The overall cost of bringing a drug to market is based entirely on who is calculating and what they are taking into consideration, but there is as yet no single defined average figure. Rates of failures, seen even in later-stage trials, are included in costs that have varied from USD1.6 billion per new drug to USD2.6 billion, USD2.87 billion and USD5 billion.

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**European Patients Forum (EPF) President Marco Greco on the future of EUPATI**
https://www.eupati.eu/advocacy/epf-president-marco-greco-future-eupati/
We live in a Europe where there are deep-seated health inequalities, where access to the most basic of care is not a given in some places, and where access to innovative medicines is simply a pipe dream. I believe that EUPATI as an EPF programme must be set in the context of wider policy debate. And the role of EPF national coalitions, together with EUPATI national platforms, in which many are partners, is to help shape a national agenda whereby genuine involvement of patients in medicines and in health policy design and delivery becomes the norm.
We are working on a full project proposal in response to an Innovative Medicines Initiative (IMI) call text of patient engagement in the life cycle of medicines. If successful, this will be a unique long-term opportunity to marry patient education through EUPATI, and patient engagement seamlessly, and create an enabling single ecosystem with patients at the forefront.
And our work continues with IMI to ensure the patients' voice in the governance and strategic orientation of IMI to 'mainstream' patient education and patient engagement in all relevant projects. An IMI stakeholder meeting will take place in October to crystalise this further. Important discussions are also taking place with

the European Medicines Agency (EMA) to optimise EUPATI's relevance in its pioneering work on patient engagement.
The global dimension is also critical. EPF is working closely with the Patient Focused Medicines Development initiative (PFMD), which we helped to found, looking at patient engagement from a global perspective, and how to enable this in other regions. There are clearly many opportunities for EUPATI's global reach and we are delighted that discussions are already underway in the U.S, Latin America and Japan. IAPO, the International Association of Patient Organization is also looking at EUPATI with its membership.
In 2012, EPF set up a capacity building programme aiming to strengthen patient organisations at all levels, through organisational development and skills building. The focus to date has been Central and Eastern Europe, and I see many opportunities in the future to build synergies between the learning outcomes here, and those of EUPATI, as both programmes advance.

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Janet Wale, HTAi PCIG
E-mail: socrates111@bigpond.com