

## HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) E-Bulletin, May 2018

*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 the May E-Bulletin

#### We are pleased to be able to use HTAi's new logo, which was released this month

The HTAi Secretariat has been working behind the scenes for months on it. Executive Director Daniel Lalonde stated: we've been working hard to build a clean, professional new identity that will see us into our next few decades. Balanced, clean, and versatile, the logo is designed to work across a variety of mediums and to support our community as we continue our great work. You'll continue to see the logo roll out across our conference app as well as various other platforms (including a new HTAi website) in the coming months.

It's a perfect time for a change and we couldn't be happier with the new look.

Looking forward to seeing everyone in Vancouver!

Daniel Lalonde

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### HTAi Matters



The full 2018 Annual Meeting programme is available online at: [www.htai2018.org/full-programme/](http://www.htai2018.org/full-programme/)

The App can be downloaded from:

<http://ios2apps.com/app/1376300195/htai-2018>

#### Sunday 3 June: Keynote Speaker is Ms. Hanna Kettler, Senior Program Office, Bill & Melinda Gates Foundation

Hannah manages the Gates-CEO Roundtable, a coalition between the CEOs of 17 global pharmaceutical and diagnostics companies and Gates Foundation leadership and the foundation's grant with Japan's Global Health Investment Fund. Her work focuses on the intersection of industry, finance, and global health & policy.

**PCIG Annual Business meeting:** at lunchtime on Monday 4 June 2018 (12.45 to 2.15 pm in Cypress 2, Level 2). We look forward to seeing you there. Lunches will be available.

#### About the PCIG Workshop

The PCIG workshop (WS13) is on Saturday 2 June: Building a shared resource of patient experience and preferences to improve HTA. Our Chair Neil Bertelsen together with Eric Low are the moderators. Karen Facey will outline how patients add value to HTA and Christine Sit of Lung Cancer Canada will talk about the burden on patient organisations in providing patient input. Different ways of tackling the issue will be introduced by a range of speakers with different backgrounds. Speakers are Lindsay Lockhart (SMC), Heidi Livingston (NICE) and Kate Morgan (Myeloma Patients Europe); Sophie Werkö (SBU, Sweden) talking about repositories and Emil Chiauzzi on PatientsLikeMe. The ideas generated will be workshopped in groups and their scopes presented to the entire group. It promises to be a lively event so we invite you to take part.

**This year's HTAI Annual General Meeting** is being held at 6:15 pm on Sunday 3 June, and is open to all Society members. We encourage you to go to Salon D to join in the discussion.

**Our PCIG Technical Officer Rebecca Addo and I have worked on a summary of patient and citizen relevant events in Vancouver – please see attachment**

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## What's Happening

**Scottish Medicines Consortium (SMC) has just launched a new website:** [www.scottishmedicines.org.uk](http://www.scottishmedicines.org.uk)

The website has a focus on increasing public understanding of SMC decisions and the health technology assessment (HTA) process. The new website includes the following series of short films, which explain various aspects of HTA.

Pharmacy Assessment Explained: [www.youtube.com/watch?v=6AnDuZTW\\_6c](http://www.youtube.com/watch?v=6AnDuZTW_6c)

Health Economic Assessment Explained: [www.youtube.com/watch?v=wtmzohhxrV4](http://www.youtube.com/watch?v=wtmzohhxrV4)

How Evidence is Considered by SMC Committee: [www.youtube.com/watch?time\\_continue=1&v=ErtvkiH2BnA](http://www.youtube.com/watch?time_continue=1&v=ErtvkiH2BnA)

Preparing a Patient Group Submission for SMC: [www.youtube.com/watch?v=ov-FYN2eXK8](http://www.youtube.com/watch?v=ov-FYN2eXK8)

The Role of Patient Group Partners at SMC Committee: [www.youtube.com/watch?v=louPilNV9z4](http://www.youtube.com/watch?v=louPilNV9z4)

The Role of the SMC Public Involvement Network (PIN) Advisory Group:

[www.youtube.com/watch?time\\_continue=3&v=z5xrgIR3icU](http://www.youtube.com/watch?time_continue=3&v=z5xrgIR3icU)

How SMC involves the Public in HTA Decision Making: [www.youtube.com/watch?time\\_continue=1&v=xYmAeclXo\\_M](http://www.youtube.com/watch?time_continue=1&v=xYmAeclXo_M)

Jennifer Dickson

Public Involvement Coordinator | Scottish Medicines Consortium

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## IPPOSI webinar on international perspectives on health technology assessment

<http://www.ipposi.ie/2018/05/08/annual-theme-2018-access-medicines-2-2-2/>

This month IPPOSI hosted a webinar for IPPOSI members that provided international perspectives on health technology assessment. You can hear how health technology assessment agencies in Canada, Sweden and Scotland are involving patients in their work and decisions. This is part of its Access to Medicines program.

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Note: The new European General Data Protection Regulation (GDPR) came into effect on 25 May 2018. The GDPR has consent requirements and organisations have been busy confirming that individuals would like to receive content from them, wherever you are.

The goal of the Patient Representatives Roundtable is to provide a platform for patient representatives to interact with other stakeholders and determine how best to engage patient representatives in the research and decision making processes.

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## European Patients Forum Position Statement on HTA in Europe

I'd like to share EPF position statement, at

<http://www.eu-patient.eu/globalassets/policy/hta/epf-position-statement-on-hta.pdf>

Valentina Strammiello

EPF Programme Manager

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**The Living with A Rare condition: The Effect on Mental Health** report that that Rare Disease UK has written is now out and may be of interest to you

<https://www.raredisease.org.uk/our-work/living-with-a-rare-condition-the-effect-on-mental-health/>

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## Patient Focussed Medicine Development (PFMD) Patient Engagement Quality Guidance launched

<http://patientfocusedmedicine.org/the-patient-engagement-quality-guidance-download/>

After almost two years of hard work, the Guidance is ready to apply in patient engagement initiatives.

The PFMD Quality Guidance is a practical tool for planning, developing and assessing the quality of patient engagement activities and projects throughout the development and lifecycle of medicines.

The innovative tool has been co-developed with more than 100 contributors, representing different stakeholder groups - including patient organisations, industry, biotech, researchers, regulators, HTA representatives, research hospitals and independent experts - through an iterative process, and building on existing PE knowledge. For background on the project and more details, please visit our website [pfmd.org](http://pfmd.org)

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### **ISPOR announces Patient Representatives Roundtable Chairs (regional) and Patient Council Members**

The Patient Council was announced earlier this year. The Patient Council will facilitate communication regionally among patient representatives, creating a global network for discussion, consensus building, and experience sharing around issues related to patient engagement in healthcare research. ISPOR Patient Council members are the chairpersons of the Society's regional Patient Representatives Roundtables.

Jan Geissler is the chair for Europe. He is founder and CEO of Patvocates, a think tank on patient advocacy, health policy, and social media, and other leukaemia based organisations. He was the Director of the European Patients Academy (EUPATI) from 2012-2017 and manages the German EUPATI platform. He is a work package leader of the new IMI2-funded EU Project HARMONY on big data for better outcomes in hematology.

Angela Chaves is the Latin American Chair. She is executive president of the Colombian Federation of Rare Diseases and is a member of the Advocacy Committee of Rare Disease International.

The newly announced chairs will be joining North American Suzanne Schrandt, director of patient engagement, Arthritis Foundation. Previously, she served as deputy director of patient engagement for the Patient-Centered Outcomes Research Institute (PCORI).

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### **Paradigm: a unique collaboration for better patient engagement in medicines development**

[www.imi-paradigm.eu](http://www.imi-paradigm.eu)

PARADIGM stands for Patients Active in Research and Dialogues for an Improved Generation of Medicines. The consortium is led by European Patients' Forum and EFPIA (European Federation of Pharmaceutical Associations) and comprises a mix of partners from patient organisations, regulatory bodies, universities, not-for-profit organisations, small and medium sized enterprises, trade associations to pharmaceutical companies.

It is a public private partnership funded by the Innovative Medicines Initiative (IMI) and EFPIA members. It was launched on 1 March 2018 and will run for 30 months, until August 2020.

The 34 partners of PARADIGM are the European Patients' Forum, EURORDIS-Rare Diseases Europe, European AIDS Treatment Group, Alzheimer Europe, AIFA, HTAi, Centre for the Advancement of Sustainable Medical Innovation from the University of Oxford, Instituto Aragones de Ciencias de la Salud, the Athena Institute from VU Amsterdam, the European Forum for Good Clinical Practices, Foundation San Joan De Deu, Synapse, the Synergist, Bayer, MSD, Merck, UCB, AMGEN, Grünenthal, GlaxoSmithKline, Janssen, Eli Lilly and Company, Lundbeck, Novo Nordisk, Pfizer, Roche, Servier, Sanofi, Novartis, Covance, Alexion, European Federation of Pharmaceutical Industries and Associations, The Association of the British Pharmaceutical Industry and Verband Forschender Arzneimittelhersteller. At the end of the 30 months, patients and researchers will have access to a framework that allows structured, meaningful, sustainable and ethical patient engagement throughout three key decision-making points of the development of medicinal products: the research priority setting; the design of clinical trials and the early dialogues with regulators and HTA bodies.

PARADIGM organised the First Open Forum in April on Patient Engagement with EUPATI (European Patients' Academy on Therapeutic Innovation) and PFMD (Patient Focused Medicines Development). The project will promote two more Open Forums on Patient Engagement that will be open to other initiatives.

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### **Publications**

The Commonwealth Fund: To The Point. Quick takes on Health Care Policy and Practice. It's the Monopolies, Stupid!  
By David Blumenthal. 24 May 2018

Drug companies enjoy government-sanctioned and -enforced monopolies over the supply of many drugs  
As long as pharmaceutical companies have uncontested market power to set prices, those prices will remain a huge problem

<http://www.commonwealthfund.org/publications/blog/2018/may/drug-monopolies-pricing>

Submitted by Russell McGowan

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**A themed section on some of the challenges in the diagnosis of rare diseases, drug approval, and patient access**  
Value in Health May 2018, Volume 21, Issue 5

[Rare Diseases: Addressing the Challenges in Diagnosis, Drug Approval, and Patient Access](#)

Kati Copley-Merriman

**Articles**

[Challenges in Research and Health Technology Assessment of Rare Disease Technologies: Report of the ISPOR Rare Disease Special Interest Group](#)

Sandra Nestler-Parr, Daria Korchagina, Mondher Toumi, Chris L. Pashos, Christopher Blanchette, Elizabeth Molsen-David, Thomas Morel, Steven Simoens, Zoltán Kaló, Ruediger Gatermann, William Redekop

[The Problem of Rarity: Estimation of Prevalence in Rare Disease](#)

Stéphane Auvin, John Irwin, Paul Abi-Aad, Alysia Battersby

[Clinical Outcome Assessments: Use of Normative Data in a Pediatric Rare Disease](#)

Dawn Phillips, Beth Leiro

[Economic Modelling Considerations for Rare Diseases](#)

Isobel Pearson, Ben Rothwell, Andrew Olaye, Christopher Knight

[Budgetary Impact and Cost Drivers of Drugs for Rare and Ultra-Rare Diseases](#)

Michael Schlander, Charalabos-Markos Dintsios, Afschin Gandjour

[Can Severity Outweigh Smaller Numbers? A Deliberative Perspective from Canada](#)

Monica Magalhaes

[Societal Preferences for Funding Orphan Drugs in the United Kingdom: An Application of Person Trade-Off and Discrete Choice Experiment Methods](#)

Siobhan Bourke, Catrin Plumpton, Dyfrig Hughes

[Evaluating and Valuing Drugs for Rare Conditions: No Easy Answers](#)

Dan Ollendorf, Richard Chapman, Steven D. Pearson

[Patient Access to Medicines for Rare Diseases in European Countries](#)

Andreja Deticek, Igor Locatelli, Mitja Kos

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