HTAi Patient and Citizen Involvement in HTA Interest Sub-Group (PCISG) E-Bulletin, May 2015

Our vision: Patient and citizen perspectives improve HTA

Welcome to this month's E-Bulletin.

I wish to thank Karen Facey and key members of the PCISG for stepping in this month to make sure that all our events at the HTAi Annual Conference go ahead as planned. This was necessary when I experienced a cardiac event and had to be hospitalised in Brussels, where I was very well looked after and experienced good participation in my health care.

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HTAi Annual Meeting Oslo June 2015

Thanks to all who responded to our call for people who are able to attend the Discussion Forum: Sunday 14 June (2 to 4.30pm): 'What would make a difference for HTA organisations and governments to involve patients in their HTA processes?' The meeting will be structured around a discussion forum and panel, facilitated by Sue Hill (present PBAC Chair in Australia and newly appointed WHO lead for HTA), with plenty of time for open discussion to identify how the HTAI PCISG can assist in the development of patient involvement in HTA through understanding: Enablers and barriers to involving patients/patient groups; What are considered to be successful models; What would assist organisations/governments in initiating and promoting this process.

And to those who expressed their intent to attend the dinner on Sunday evening, 14 June 2015 in Oslo to celebrate the 10th Anniversary of the PCISG.

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Call for 'Expressions of Interest' for the PCISG Steering Committee and a new Vice Chair

This has been extended to the end of June, so if you have been considering the possibilities now is the time to act.
If you like what we are doing, or have an alternative positively constructed viewpoint, this is the time for you to working more closely with us 'in your spare time'.
Expressions of interest are to be sent to Tara Blasco Raj (E-mail: tblascoraj@htai.org) and will be followed up with a formal process.
Due date: Tuesday 30 June 2015

The objectives of the PCISG are available at:
http://www.htai.org/interest-sub-groups/patient-and-citizen-involvement/pcisg-home/pcisg-objectives.html

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The NIHR Horizon Scanning Research & Intelligence Centre (HSRIC)

HSRIC has recently published two documents relating to the patient and public involvement and engagement (PPIE) work at the Centre: a report of PPIE activity from 2012-2014 and a PPIE strategy for 2015-2016. Both of these reports can be accessed on HSRIC's website at: http://www.hsc.nihr.ac.uk/about-us/governance/

Kathryn Miles, NIHR Horizon Scanning Research & Intelligence Centre
University of Birmingham

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McMaster Health Forum

The McMaster Health Forum, with support from the Labarge Optimal Aging Initiative, recently hosted a public talk to examine the latest evidence on breast cancer screening and the impact this evidence has on physicians and their interactions with patients. Click here to read the summary or watch the video from this talk (http://www.mcmasterhealthforum.org/new-at-the-forum/2015/04/17/making-sense-of-the-evidence-on-breast-cancer-screening).
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"I don't feel that I have the ground to stand on to say 'do not screen' – I think that's the wrong message," said Dr Risdon. "But what I do feel that I can say now is that choosing not to screen is very sane and can be very consistent with caring for your health and I would strongly support either choice depending on what a woman felt was going to be best for her."
"Not all cancers are the same," said Dr Sussman. "I think we have to do a better job of characterizing what is truly something that is a threat to somebody's health versus something that develops that we're just finding."

Dr. Cathy Risdon is a Professor and Associate Chair, Academic with the Department of Family Medicine at McMaster University and is a Co-Director of the McMaster Family Practice. Dr Jonathan Sussman is an Associate Professor with the Department of Oncology, McMaster University and a Radiation Oncologist at the Juravinski Cancer Centre in Canada.

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http://www.mcmasterhealthforum.org/docs/default-source/Product-Documents/citizen-panel-summaries/nutritional-risk-and-older-adults-in-ontario_cps.pdf?sfvrsn=4

The Forum recently released a citizen panel summary, which identifies some potential solutions to addressing nutritional risk among older adults. Among the options identified, the most promising focused on enhancing the coordination, integration and monitoring of services for older adults at nutritional risk.
The citizen brief and the citizen panel it was prepared to inform were funded by McMaster University's Labarge Optimal Aging Initiative and by the Government of Ontario (through a Ministry of Health and

Long-Term Care Health System Research Fund grant entitled Harnessing Evidence and Values for Health System Excellence

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National Institutes of Health (NIH) Precision Medicine Cohort, USA
http://grants.nih.gov/grants/guide/notice-files/NOT-OD-15-096.html

This Request for Information (RFI) sought feedback to help guide the National Institutes of Health (NIH) in creating a longitudinal cohort of 1 million or more Americans who have volunteered to participate in research as part of the President's proposed Precision Medicine Initiative. Participants will be asked to give consent for extensive characterization of biologic specimens (potentially including cell populations, proteins, metabolites, RNA, and DNA whole-genome sequencing, if/when costs permit) and behavioral and environmental data, all linked to their electronic health records (EHRs). Qualified researchers from many organizations will, with appropriate protection of participant confidentiality, have access to the cohort's de-identified data for research and analysis.

Opportunities for evidence-based precision medicine have greatly expanded with the development of better large-scale biologic databases and computational tools, among other things. The goals of the NIH Precision Medicine Cohort are to enable better assessment of disease risk, understand disease mechanisms, and predict optimal therapy for a broad range of diseases through the study of a large group of people who have volunteered to provide data and biospecimens over time to a cadre of researchers pursuing these research goals. These data will also enable observational studies of drugs and devices and potentially prompt more rigorous interventional studies that address specific questions.
Characteristics of such a large-scale study that might maximize its research value may include: 1) a sufficiently large number of participants to achieve adequate power for common disorders and reasonable representation of rare disorders; 2) intentional over-sampling of populations underrepresented in research to permit meaningful inferences about these groups and to study health disparities; 3) a broad age range to provide information on disorders from infancy to old age; 4) a broad range of genetic backgrounds and environmental exposures; 5) a broad array of clinical and laboratory information, not limited to any single disease, as well as patient reported outcomes; 6) sophisticated dietary, other lifestyle, and environmental exposure assessment, preferably provided directly from participants using mobile devices and wearable sensors; 7) access to comprehensive electronic health data on participants for baseline, follow-up, and possibly also retrospective (prior to study entry) data collection, as well as return of actionable results for use in their clinical care; 8) return of appropriate information and results to participants as they desire; 9) collection and storage of biological specimens; 10) access to study data and biologic materials to qualified researchers to empower research on many diseases by researchers in many sectors; 11) community engagement in the design and implementation of the study, including a state-of-the-art consent process, to allow multiple uses of the data, regular feedback to participants about findings and progress; and 12) a study design that ensures a high follow-up rate.

Background Information
On January 20, 2015, President Obama announced a new Precision Medicine Initiative for fiscal year 2016 in his State of the Union address, and expanded on the announcement at a White House event on January 30, 2015. On that same day, Drs. Francis Collins and Harold Varmus published a Perspective in the New England Journal of Medicine, "A New Initiative on Precision Medicine." Opportunities and

obstacles for developing such a cohort were explored in a preliminary way in a February 2015 NIH workshop, "Building a Large U.S. Research Cohort."

Information Requested

The NIH seeks information on characteristics, purpose, or other overall aspects in the development and implementation of a large U.S. precision medicine cohort. Information is also sought regarding existing and potentially new entities that have the capability to identify and follow ideally 10,000 or more participants and, if combined with other research entities, could comprise a longitudinal cohort of 1 million or more Americans. The participants should consent to joining this large U.S. cohort and provide their medical, genomic, and other health-related data, with appropriate protections, for broad research use.

Participants should be accessible for consent or re-consent for data sharing, whole genome sequencing and other biologic measures, multi-use (ability to perform analysis of multiple traits and measures, not just one single disease), and call-back for consent for further in-depth study.

The NIH seeks comments on any or all of, but not limited to, the following topics:

A. General topics on the development and implementation of this large U.S. cohort.

1) The optimal study design and sample size for a large U.S. precision medicine cohort.
2) Data to be collected at baseline and follow-up, including mode of collection and frequency and length of follow-up.
3) Potential research questions that could be uniquely or more efficiently and effectively pursued in a large U.S. precision medicine cohort.
4) Any other suggestions for NIH to consider in the development and implementation of such a research cohort.

B. Suggestions for existing or potentially new research entities (a health care system, research network, cohort study or consortium, or other entities such as longitudinal studies using digital-based platforms) **that might be combined into a large U.S. cohort. Providing the following information would be useful when suggesting research entities.**

1) The capability of the existing or potentially new research entity to efficiently identify and follow 10,000 or more participants who are likely to consent to providing their medical and other health-related data, biospecimens, and genomic data for broad research use, including in sub-group analysis that could help assess various treatment effects and outcomes. It would also be useful to provide the rationale that potential participants are likely to consent, as well as experience with and ability to participate in central IRB and a master contract agreement to streamline enrollment of the precision medicine cohort.
2) The capability for the research entity to provide individual-level participant data, particularly those from electronic health data (including both electronic health record and payer data), that can be integrated into a standard format to create a combined large longitudinal precision medicine cohort.
3) The capability for the research entity to track and retain the participants for several years of follow up. The race/ethnic composition, sex, and age distribution of participants from the research entity likely to consent, by standard U.S. Census categories, would also be helpful. The NIH especially seeks information about studies of populations underrepresented in research and those with phenotypes or disorders of high public health and human impact. Additional information that would be of use includes: for health care systems, the current patient turnover rate and efforts that can be made to capture longitudinal data from clinical visits outside of the system and to continue follow participants who leave the system entirely; and for ongoing cohort studies, the retention rate to date.

Responses
All responses were due in early May. The RFI is for planning purposes only and the United States Government will not pay for the preparation of any information submitted or for its use of that information.
Responses will be compiled and may be shared publically.

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Patient Group activities

European Patients' Forum (EPF) launches a one-year campaign on Patient Empowerment
EPF launched its campaign *on*20-21 May EPF will launch our one-year campaign dedicated to Patient Empowerment with a conference in Brussels, Belgium*: "We want to build the momentum on the empowerment of patients in Europe to contribute towards genuine sustainability of quality health systems and societal 'growth'."*
"*We want to be full partners in the management of our conditions according to our individual capacities and situation. We need to be empowered to do so. Empowerment starts with tailored high-quality information and health literacy, to enable us to make informed choices about our treatment and care."*

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The 27th edition of the Drug Information Association (DIA) EuroMeeting, 13-15 April, entitled "Development, Innovation, Access and Patient Safety"

Four major issues for the European Patients Academy (EUPATI, were presented and discussed:

- A good model of participation and interaction with patients and patient organisations has already been developed about engagement with regulators by the European Medicine Agency, presented by Isabelle Moulon.
- According to Matthias Gottwald from Bayer, pharmaceutical industry codes are gaining momentum due to the intense collaboration carried out within the European Patients' Academy structure itself and are rapidly moving towards a more patient-focused approach.
- The Health Technology Assessment (HTA) framework has shown a long history of patient involvement although many of the advancements are in preliminary stages, according to Karen Facie from HTA International.
- A big challenge remains in getting the patient into ethics committees. Due to a lack of regulations and of a proper structure where experiences can be exchanged between countries the situation is very fragmented and the debate to establish a common framework of interaction is still in the early stages. "This remains one of the biggest challenges for patients", according to Andrea Heckenberg from the EUPATI Ethics Panel.

From European Patients' Forum Newsletter (April 2015)

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Congratulations to Eibhlin Mulroe who has left IPPOSI to take over as the CEO of the All Ireland Cooperative Oncology Research Group (ICORG), which is a cancer trials group in Ireland (www.icorg.ie/).

Eibhlan took over as the first CEO of IPPOSI in July 2007, which has grown and developed networks of stakeholders both in Ireland and the EU. I am very proud to have had the opportunity to represent the

patient perspective and even more proud to call many of you my friends. She has strongly supported the work of EUPATI.
EUPATI aims to make a visible difference for patients in areas of medicines development, safety of medicines and access to treatments. It is organised on a national basis across 12 countries in Europe and the National Teams comprise members from patient organisations, academia and industry.
EUPATI held a successful Workshop in Dublin this year, which drew in 180 delegates from 26 countries.
Later this year EUPATI will launch the EUPATI Patient Advocate Toolkit, and very soon after will begin work on the Online Resource Library for the health-interested public.

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Cittadinanzattiva: the Italian Active Citizenship Network

The Cittadinanzattiva's representative office is to be opened at the European Union in Brussels. This marks an important historical moment in the 37 years of the organization. The operation was conducted personally by the Secretary General Antonio Gaudioso, who has followed-up on his statement at the National Congress of Chianciano Terme in June 2012 that "Europe is our home and our future, and it has extraordinary spaces to enhance thirty years of work of our organization and, at the same time, open our minds by comparing it with the best civic experiences from other countries". (Active Citizenship Network (ACN) Newsletter n. 29)

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Civil society statement on PrEP for HIV prevention in Uganda

Uganda, and indeed the rest of the world, is at a pivotal point to change the trajectory of HIV/AIDS. More people in need of life-saving treatment have been enrolled, the number of prevention options has increased and there has been increased funding over the last decade or so. But this is only the headline. Reports indicate that Uganda's incidence is now rising in some populations. Retention data tell us that some people who have been enrolled on treatment are not receiving the support they need to remain adherent.
With this backdrop, civil society reiterates the fundamental importance of antiretroviral treatment for people living with HIV as the essential, non-negotiable component of the country's HIV response.
At the same time, funding for treatment and prevention and is under real threat, among many other challenges.
At the same time, we must use every available scientifically tool to prevent new infections. This is why, even as we recognize and affirm the central priority of strengthening and expanding Uganda's ART program for people living with HIV, we also urge the country to swiftly commit to a concrete set of actions that will answer the question of whether daily oral PrEP using TDF/FTC fits into the Ugandan prevention strategy which must also continue to include HIV counseling and testing, voluntary medical male circumcision, STI treatment, behavioral interventions, and other proven options for key populations.
The data are clear: daily oral PrEP works if you take it. Recent clinical trials conducted in several countries, including Uganda, have shown clearly that daily, oral pre-exposure prophylaxis (PrEP) using the antiretroviral drugs tenofovir and emtricitabine dramatically reduces the risk of HIV infection for men and women who take it as directed. While PrEP won't be right for every individual at risk for HIV, a big number of men and women at high risk of HIV infection will benefit from it – if they can access this potentially life-saving intervention. It's however important to note that PrEPis integrated into comprehensive, high-impact prevention programs for all people at risk, with particular attention to

discordant couples, young women and married women who continue to bear the brunt of the epidemic, and key populations.
Daily oral PrEP could be a key tool for many populations, including people living in HIV serodiscordant relationships, sexually active young women and other key populations are at high risk of infections. Right now oral PrEP is the only woman-controlled method available that is not linked to use at the time of sex. Second, is that access to Truvada—the drug used in oral PrEP— is a fundamental concern for and need of people living with HIV/AIDS. For example, HIV serodiscordant couples need options that can help them during periods of increased vulnerability like when they want to have a baby, when there is condom fatigue and when the HIV infected person has not achieved viral suppression.
PrEP has great potential relevance. It is also a complex intervention and a niche strategy. It is not for everyone all the time. But Uganda cannot answer the question of if, where and how daily oral PrEP fits in without taking concrete action. The data to date are not enough. It is therefore critical that the government work with implementers, civil society groups and others to actively explore this question. Specifically:

- we request the MOH and UAC to develop prescriber guidance that can ensure safe use of PrEP – which is based on an existing drug that is available in Uganda for those who can afford it – such that the general population has correct information while Uganda's investigation into the role of daily oral PrEP is underway.
- We request that the national ART committee work with other relevant stakeholders, particularly civil society, to develop a plan for investigating PrEP's utility, relevance and cost effectiveness in the Ugandan context—with the plan prepared in the next three months, and implemented over the next 12 months to address the question: *Is there a role for daily oral PrEP using TDF/FTC for prevention in Uganda?*

As leaders of the International AIDS Society said in a commentary in the Lancet this month, "Now that PrEP has been proven to work, we believe that PrEP is not only sound public health policy, but also a human rights imperative." As Ugandan civil society, we couldn't agree more.

Joshua Wamboga, Uganda

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Further reading

Lopes, E., Carter, D., & Street, J. (2015). Power relations and contrasting conceptions of evidence in patient-involvement processes used to inform health funding decisions in Australia. *Social Science & Medicine,* 135, 84-91.
This paper will be accessible free of charge on this link (until 4th July 2015):
http://authors.elsevier.com/a/1R1Q8-CmUV7kA

In the current issue of International Journal of Technology Assessment in Health Care:

Tarang Sharma, Moni Choudhury, Bindweep Kaur, Bhash Naidoo, Sarah Garner, Peter Littlejohns and Sophie Staniszewska. Evidence informed decision-making: the use of colloquial evidence at NICE.
http://journals.cambridge.org/action/displayAbstract?fromPage=online&aid=9698629&fulltextType=RA&fileId=S0266462314000749

Marie-Pierre Gagnon, Marie Desmartis, Johanne Gagnon, Michèle St-Pierre, Marc Rhainds, Martin Coulombe, Mylène Dipankui Tantchou and France Légaré. Framework for user involvement in health technology assessment at the local level: views of health managers, user representatives, and clinicians. http://journals.cambridge.org/action/displayAbstract?fromPage=online&aid=9695940&fileId=S0266462315000070

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Correction:

OHE is the Office of Health Economics (OHE)and it is London-based. David Grainger, Global Public Policy Director, Eli Lilly and Company, has recently joined their Policy Advisory Board, which comprises a number of prominent health economists and about 3 industry 'experts' (the others are from within the UK industry).

A series of three articles exploring future expectations for new drugs of evidence of relative effectiveness in Europe and comparative effectiveness in the USA in 2020

The Office of Home Economics publications are available from the link: https://www.ohe.org/news/ohe-publishes-journal-comparative-effectiveness-research-future-expectations-evidence-relative

OHE provides authoritative resources, research and analyses in health economics, health policy and health statistics both through independent research and consultancy. Our work informs decision making about health care and pharmaceutical issues at the UK, regional and international levels. OHE has diverse and extensive experience in the private, public and charitable sectors. Each individual maintains the highest professional standards in both working style and project results.

The OHE's current work programme is supported by research grants and consultancy revenues from a wide range of UK and international sources: the Association of the British Pharmaceutical Industry (ABPI) and other commercial clients, the Department of Health Policy Research Programme (PRP), the National Institute of Health Research (NIHR), the Medical Research Council (MRC), the EuroQol Foundation, and a number of charitable and other organisations.

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Janet Wale, Chair HTAi PCISG
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I am happy to address any questions you may have as a result of this e-Bulletin, and to forward information.