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

HTAi RICC Interest Group:

Regulatory Interactions & Conditional Coverage

a new Interest Group formed from the merger of the HTA-Regulatory Interactions IG and the Coverage with Evidence Development IG

Newsletter, 10 Oct 2016

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1 Update on HTAi Rome 2017 – time to start preparing submissions

This month the call for abstracts to the HTAi 2017 Annual Meeting in Rome has commenced. The deadlines are:

- **Workshops & Panels: December 2, 2016**
- **Poster & Oral Presentations: January 16, 2017**

We encourage RICC members to consider participation in the conference programme on topics relating to HTA-Regulatory Interactions and Conditional Coverage. Please be careful to review the abstract submission guidelines in detail prior to submission because this year there have been some changes to the submission process and scoring system. In addition, we expect the number of abstract submissions, especially for panels, to far exceed the number of available places and so it will be important to ensure your abstracts are of high scientific quality and relevance to the society. In addition, be careful to consider the 'bonus' factors relating to scoring as in previous years these became key differentiators in a closely contested selection process.

Please also note: as with all interest groups, **the RICC Interest Group will be allocated two abstract submissions that can be designated as formal submissions on behalf of the IG**. Such submissions still have to meet the scientific quality thresholds (as is appropriate) but will obtain a 'bonus' in the final selection process. Following discussion in the Annual Business Meeting, we would like to develop two 'official' panels.

If you are interested in participating in the development of the panel concepts as defined in

the ABM minutes or if you have an alternative idea for a panel that you believe might be of high importance to the RICC membership, then please contact Urs and myself. If we have more than two panel proposals to select from, then we will organise a vote via survey monkey for the membership to choose which two to initially allocate as our two 'formal' submissions but we will undertake to help support development of all panels that would represent the RICC.

HTAi 2017 abstract information and guidelines: link

HTAi 2017 abstract submission page: link

2 **Minutes of the RICC 2016 Annual Business Meeting**

This year's Annual Business Meeting, the first for the new RICC Interest Group, was held on Friday, May 13 2016 during the HTAi Annual Conference in Tokyo. The meeting was well attended and the participants were engaged in the discussion.

The ABM commenced with the official launch of the RICC Interest Group, a discussion of the Terms of Reference, including the objectives, scope, governance and methods of working. Current activities were reviewed, in particular, changes to the website and repository. Following this, a brainstorming discussion focussed on future activities of the RICC, with a particular emphasis on development of panel proposals for the coming 2017 annual HTAi meeting.

The full minutes have been posted to the RICC website and can be downloaded here

3 **RICC Panel at Tokyo HTAi Meeting**

For the 2016 Annual Conference, the RICC Interest Group developed a panel that brought together the topics of HTA-Regulatory Interactions with Conditional Coverage to explore the issue of how a changes to regulatory approval pathways in the US and Europe could have a flow on effect to HTA in the Asia-Pacific and how such HTA bodies could respond to the potential increase in uncertainty. The panel title was: *How can HTA's in the Asia-Pacific respond to increased clinical uncertainty as a consequence of expedited US and EU regulatory processes? Is risk-sharing the answer?* The panel was chaired by Franz Pichler (RICC chair) and the panellists were Dr. Michelle Mujoomdar (CADTH, Canada), Professor Arnold Chan (HDRC, Taiwan) and Professor Adrian Towse (OHE, UK).

Amanda Cole (OHE) developed a report on the panel which has been approved by all of the panellists. The panel report has been posted to the RICC website and can be downloaded here

4 **News: Latest developments at NICE: the Cancer Drugs Fund**

As part of NHS England's new approach to the appraisal and funding of cancer drugs in England, the National Institute for Health and Care Excellence (NICE) has made some changes, which are described in an addendum to its methods and processes.

NICE's appraisal process for cancer drugs now starts much earlier with the aim of publishing draft guidance before a drug receives its marketing authorisation. And NICE can make an new type of recommendation that provides a fast-track route to NHS funding: a treatment can be recommended for use in the Cancer Drugs Fund (CDF) when there is plausible potential for it to satisfy the criteria for routine commissioning, but there is significant clinical uncertainty.

When a treatment is recommended for use within the CDF, a managed access agreement will be drawn up. This consists of a data collection arrangement and a commercial access

agreement.

The data collection agreement outlines the key areas of uncertainty and explains how they will be addressed through data collection. NICE's specification for CDF data collection arrangements describes two main options for collecting data. The first is ongoing or new clinical studies. The second is 'real-world' data collection using the Systemic Anti-cancer Therapy (SACT) dataset, which is potentially complementary to clinical studies as well as being a standalone option.

The commercial access agreement determines the level of reimbursement during the data collection period. This will reflect the decision uncertainty: greater uncertainty will result in a lower level of reimbursement. Companies will receive less for the drug in the CDF than if it had been recommended for routine commissioning.

At the end of the managed access period, NICE will re-appraise the drug to decide whether the drug can be recommended for routine commissioning.

Contributed by Linda Landells (NICE)

5 News: Adaptive pathways controversy

On the third of August, 2016, the EMA published its final report on the adaptive pathways pilot which had commenced in March 2014. The EMA emphasised that this approach was not intended to be a new mechanism, was applicable only to a subset of new medicines and that the focus was on prospective planning for development so as to incorporate requirements from multiple stakeholders and additionally to develop evidence in an iterative manner, including post authorisation use of real world data to supplement RCT evidence. The report acknowledged that there were outstanding issues that had not been resolved during the pilot phase, in particular in relation to use of real world evidence pilot. The report proposed future discussions, including an upcoming EC-EMA multi-stakeholder adaptive pathways workshop on 8 December at the EMA in London.

On the 9th of August, IQWIG issued a press release "Adaptive pathways: EMA still leaves open questions unanswered." The core of the IQWIG concern is that '*evidently neither industry nor EMA has a concept as to how real world data can be used after drug approval to allow drawing reliable conclusions on benefit and harm*.' They argue that without any clarity on how to use RWD/RWE that the EMA should stop with the adaptive pathways until there has been an open public debate on exactly how real world data can be generated and real world evidence used in a scientifically valid way.

On the 11th of August, EMA responded to the IQWIG criticism in an interview with the regulatory magazine Pink Sheet. According to the article, the EMA has dismissed both the IQWIG's claims about the limitations of real world data and also their call for the EMA to stop accepting applications to the adaptive pathways programme. The article outlines the EMA responses to the specific IQWIG concerns.

The controversy has continued with the publication on 28 September, in Frontiers of Pharmacology of a paper "Payer's views of the changes arising through the possible adoption of adaptive pathways" by Michael Ermisch (National Association of Statutory Health Insurance Funds, Berlin) and others. Highly critical of the adaptive pathways concept and the EMA, the paper comprehensively elucidates the core concerns of the payer community in a single document. This will no doubt be useful for both sides of the debate.

Meanwhile ADAPT-SMART, the Innovative Medicines Initiative (IMI) consortium on adaptive pathways, also issued a press release on the 16th of August, welcoming the EMA report as a step forwards, in particular, in demonstrating the feasibility of bringing together diverse stakeholders for the purpose of developing a prospective plan for generating data across a medicines lifecycle. The press release also noted the need for more robust methodologies for the assessment of real world evidence.

Of relevance to this controversy, the IMI project GET-REAL is holding its closing meeting on 24 November in Brussels "Delivering tools for real world evidence development" during which the presenters will discuss the outcome of the three-year project that was aimed at improving real world evidence methodologies.

Final comment: We believe that the controversy around adaptive pathways and the use of real world data could make an ideal panel discussion for Rome 2017 – Urs Brügger and I will be looking to develop a panel for the RICC on this topic and invite any RICC members who might be interested in this panel to join us in a working group.

6 Final Comment

As always, we are very eager to hear your feedback as that is the best way we can ensure that this Interest Group remains….interesting. We want to meet your needs and so do let us know how we can improve our work.

If you have project or conference panel/workshop ideas that might be suitable for the RICC, especially if you would be interested in contributing a little bit of your time, then do let us know.

Thank you all for your interest in and support of the RICC IG.

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