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

HTAi RICC Interest Group:

Regulatory Interactions & Conditional Coverage

Special Focus on CED

Newsletter, 17 May 2017

Contents

1 HTAi Rome 2017 & RICC Annual Business Meeting

The Annual HTAi conference is almost upon us – the best annual opportunity for the HTA community to come together to share ideas and learnings. This year's programme is packed with interesting workshops, plenaries, panels, oral and poster presentations including several of interest to the RICC. The draft programme has just been published on the conference website: link

The RICC annual business meeting will take place at 07:00-08:30 on Tuesday 20 June. There are no conflicting sessions at that time and a light breakfast will be provided. The annual business meeting is an important opportunity for interest group members to review the activities of the group, to propose new topics, activities or to initiate work streams for the coming year.

During the 2017 annual business meeting there will be two key events

1. The election of a new Executive Team (Chair/vice chair or co-chairs) – see section 2 below

2. A discussion on the use of real world evidence in HTA for adaptive pathways and conditional coverage lead by **Alric Ruether** (IQWiG) and **Carole Longson** (NICE)

2 Election of a new Executive Team for the Governance of the RICC IG

Both Urs and myself are planning to step down from our positions as chair/vice-chair of the RICC this June. The HTAi Secretariat is assisting in establishing an Executive Team

replacement and JoAnne Zaborowski recently distributed a call for expression to the RICC membership. Below is the relevant excerpt from that email:

> "This is an exciting time for HTAi's 10 Interest Groups as the Board is actively engaged in infusing more resources into the activities of each IG. The Board has identified some pivotal activities specific to your Interest Group in line with our current Strategic Plan available for your review here: http://www.htai.org/htai/strategic-direction.html
>
> We are cordially inviting you to put forward your formal Expression of Interest in being a member of the Executive Team by sending an email initially to me (HTAi IGs Coordinator) by the **May 15.** Guidance and support will be provided by the HTAi Secretariat and the Chairs of the Interest Group Steering Committee as need be. If you are interested in this opportunity, and have any specific questions about the activity of this IG please contact Franz Pichler at: fpichler@lilly.com or the HTAi Secretariat at your convenience and we would be very pleased to provide you with more information.
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> **Please note:** You must have been an official member, in good standing of the Society for the past 2 years, in order to be part of this Executive Team. If you wish to renew your membership for 2017-18 (June 1, 2017-May 31, 2017), please do so: https://www.xcdsystem.com/htai/member/ "

3 An update of CED programmes in Germany and the Netherlands

In 2014, I did a survey of Coverage with Evidence Development (CED) programs in 10 different countries. The report can be found in the RICC repository: link to report

One of my conclusions was that CED had gone through three phases. There was an early first phase roughly ten to fifteen years ago. At that time, CED was seen in many countries as a panacea for all decision problems in HTA when a promising new technology came to the market, yet there was a lack of evidence. Then phase two came as a disillusionment with the CED approach. Many CED programs simply did not work. So phase three started as a relaunch of CED programs with a stricter approach and clear criteria when CED was "indicated" and when it was not.

Germany and the Netherlands were at the forefront of phase three. Both countries started new CED programs around three years ago. Therefore, it will be interesting to learn where these two countries stand with their CED programs. Matthias Perleth from the GBA in Germany and Hedi Schelleman from the Zorginstituut Nederland give an update on those two CED programs.

Urs Brügger, RICC Vice Chair

Coverage with Evidence Development (CED) – Implementation of new regulations for benefit assessment of medical device-related procedures in Germany

In 2012, a new coverage with evidence development scheme to assess the benefit of diagnostic and treatment methods was implemented in Germany. This new regulation (section 137e in the Social Code Book V), for the first time, offers the opportunity for the Federal Joint Committee (G-BA) to initiate testing directives (CED trial) for methods with yet unproven benefit, which offer the potential of being an adequate treatment alternative. Besides, this new regulation includes the opportunity for manufacturers of diagnostic or treatment methods for which medical devices are decisive to apply for CED trials if they

agree to pay the study overhead costs. Before this new regulation, only the stakeholder organisations of the G-BA - the highest decision-making body of the joint self-government of physicians, hospitals and health insurance funds - were able to initiate benefit assessments.

Up to now, however, only two directives for CED trials (stem cell transplantation for multiple myeloma and magnetic resonance-guided focused ultrasound surgery for uterine myomas) have been issued by the G-BA and will be commissioned soon.

It has emerged that the underlying decision-making process is more time consuming than expected due to several reasons. One prerequisite for conducting a CED trial is that the method shows potential for additional patient-relevant benefit compared to current standard. This preliminary evaluation is based on the available evidence and further information, which is being gathered by manufacturers, medical societies and other experts by means of time-consuming written and oral hearings. Only once a year, the most promising examination and treatment methods are selected for a CED trial. Thus, depending on the complexity of the required methodology, it may take up to three years from application until the final resolution can be published. However, the most intricate requirement why CED trials could not be realized yet is that concerned manufacturers of the decisive medical device refuse to pay their share of the study related (so-called overhead) costs.

In 2016, a second regulation involving CED became effective (§137 h SGB V). It relates to the assessment of inpatient methods which involve the use of medical devices which are supposed to be „highly-invasive“ (i.e. active implantable, active class IIb and invasive class III). This regulation has been linked to the so-called NUB-procedure (NUB: New methods for treatment and diagnosis), which is a payment scheme for remunerating cost-intensive, innovative services and technologies that are used in addition to the procedures included in the valid DRG case-based flat rate. The NUB-procedure is only open to technologies / procedures that are considered new in Germany. Once a year, hospitals can file electronic requests to the Institute for the Hospital Remuneration System (InEK) to enquire whether the conditions for negotiations have been set for hospital-specific temporary extra-budgetary payments (NUB-payments). Under the regulation of section 137h SGB V, hospitals additionally have to file an electronic request if the method in question fulfils the criteria of using a “highly invasive” high-risk medical device. The G-BA then has to verify if the submitted information encompasses the first NUB- application for this exact diagnostic or therapeutic method, if the medical device involved belongs to class IIb or III and is “highly invasive”, and if the new diagnostic or therapeutic method provides a new theoretical scientific concept. Only if all three aspects apply, an assessment process will be initiated with a subsequent G-BA decision regarding coverage.

Up to now, the G-BA decided on eight methods under the §137h SGB V regulation. Six methods showed no potential because either the available evidence was not considered sufficient or no data was available at all. In consequence, these methods have to be excluded from reimbursement. For two methods, the available data was sufficient to assign positive potential. Corresponding to the procedure in § 137e SGB V, CED directives have to be developed. However, within § 137h SGB V, the given time frame, until the trial has to be finished, is two years. It remains to be seen whether this requirement expedites the process and thus contributes to the use of safe and effective devices.

Contributed by Alexandra Nolting (MSc Epi), Dr. Nina Egger, Dr. Rebecca Muckelbauer, Dr. Matthias Perleth (MPH), Medical Consultancy Department, Federal Joint Committee, Berlin, Germany

Update of the Dutch CED programme

In 2012 the Dutch Ministry of Health started a new CED program. Under the CED programme, a promising intervention(1) is funded through the health care system (health insurance) despite insufficient evidence about the effectiveness. The mandate for CED is stated in our law, which means that each intervention that falls under the CED needs to be separately listed. This can be done each 3 months. The research can be funded through a public health research body (ZonMw) or the manufacturer.

In the Netherlands CED is done to generate effectiveness data and cost-effectiveness data. To date, 14 interventions have fallen under the CED program. Zorginstituut Nederland and ZonMw monitor the research. At the end of the CED period, Zorginstituut Nederland will re-appraise the intervention to decide whether the intervention will be paid without conditionals through the health care system. All completed research (n=3) gave an answer on our 'evidence gap'. However, only one intervention had a positive risk-benefit balance (namely intra-arterial thrombolysis / thrombectomy).

The Dutch minister of health has evaluated the CED program and concluded that the CED program would probably function better as a tax-financed system (e.g., like in England). Therefore, in the next coming months, the Dutch CED will most likely change.

For more information about:

- the Dutch CED program: link,
- and interventions that fall under the CED program:link.

Hedi Schelleman, Zorginstituut Nederland

4 Outcomes-based contracting in the US: the Genentech-Priority Health pilot

At present in the US, there is considerable interest in outcomes-based (or 'performance-based') contracting, which are in some ways analogous to CED in the sense that following patient access to the intervention, additional clinical evidence is collected in order to provide more certainty around the intervention's impact. In the US setting, this is usually based on real world observational data rather than additional clinical trials run in parallel to patient access. While there is diversity in the types of outcome-based contracting, in general they impact pricing via the level of rebate paid by the manufacturer to the insurer which will be linked to predefined patient outcomes.

Writing in Health Affairs, Marc Watrous, senior vice president of managed care and customer operations at Genentech, and John Fox, associate chief medical officer at Priority Health, discussed early lessons from the Genentech-Priority Health outcomes-based pilot, which used Genentech's Avastin (bevacizumab) in patients with non-small cell lung cancer. Under the agreement, the companies tied rebates to progression-free survival, which was a key endpoint in a Phase III study. The authors said the pilot was successful in that both companies learned how to overcome operational, clinical and contractual obstacles, and they showed this kind of agreement is feasible. The companies measured the success of the pilot in operational learnings, rather than immediate financial benefits, and the authors said outcomes-based contracts could help align value and cost.

Health affairs blog (April 3, 2017): link

1 In 2012 only intramural intervention could fall under the CED program, but now extramural intervention can also fall under the program.

5 Final comments

Both Urs and I have greatly enjoyed our roles as chair and vice-chair of the RICC IG and are both stepping down for similar reasons – a changing focus of our respective jobs as well as the recognition that it is time for this interest group to have fresh leadership. If any of you are interested in finding out more about the new Executive Team then please do contact either of us, or JoAnne Zaborowski, the HTAi Interest Groups Coordinator (jzaborowski@htai.org)

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