HTAi 2017 Report

Towards An HTA Ecosystem: From Local Needs To Global Opportunities

17–21 June 2017

www.costellomedical.com

costello medical consulting

HTAi 14th Annual Meeting, Rome, Italy

An ecosystem was defined as an environment that contains interconnecting and interacting parts. As an organisation, HTAi want to lead the way in connecting the different components of the HTA ecosystem, including researchers, decision makers, patients, regulatory bodies, guideline organisations and industry. This conference focussed on how these different stakeholders interact and how they can together work towards a more integrated system for efficient HTA.

The following key themes of the conference are detailed in this report:

Integrated HTA Processes

The integration of HTA processes, whether between countries or within the life cycle of a technology, was a key theme at this year's conference. The first plenary session looked back at how HTA has developed over the years, from writing reports in 2005 with limited stakeholder interaction and using randomised controlled trial evidence only, to more recently focusing on communication, stakeholder involvement and external processes.[1]

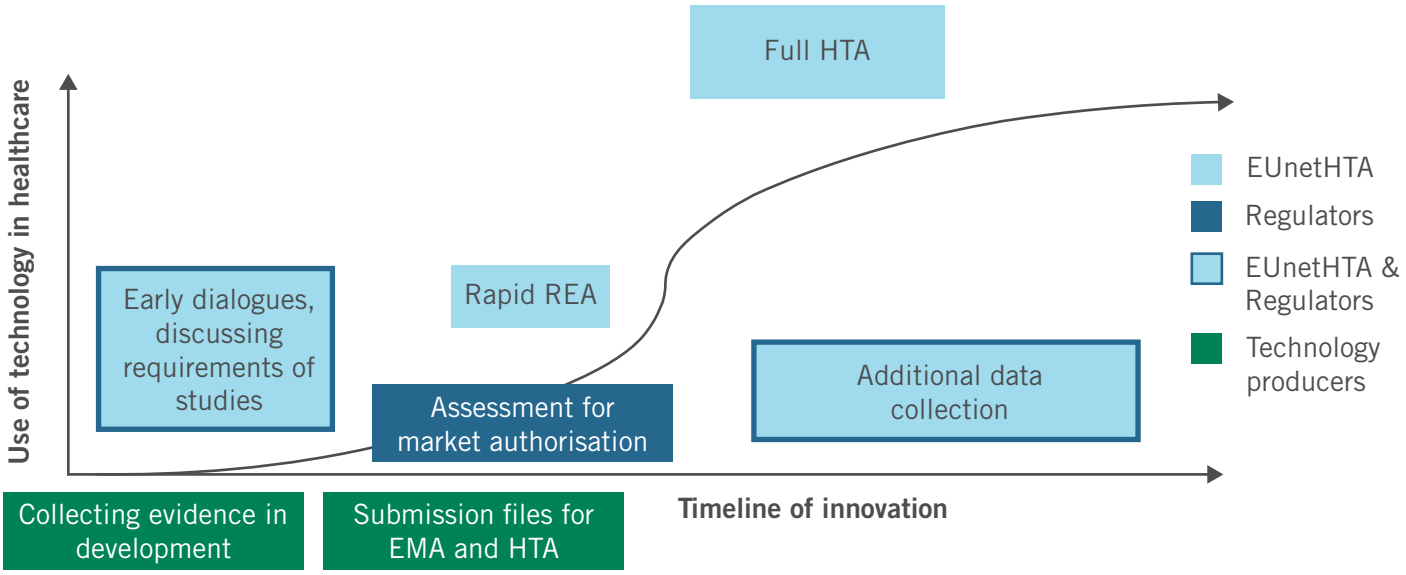
Early dialogue as a means to integrated HTA was widely discussed:

- Dialogues between manufacturer's and regulators/ HTA agencies could occur simultaneously to ensure pivotal trials collect data on endpoints required for both processes
- A single regulatory/HTA process was discussed, although the general consensus was that this may not be the right approach, and instead the focus should be on working in parallel
- Early dialogue should enable better data collection, which could take the form of global data for local output
- Collaboration and integration should occur throughout the life cycle of a technology, especially now that more types of technology are available, not just pharmaceuticals and medical devices

There was much discussion around EUnetHTA, which is currently in the second year of its Joint Action 3 (2016–2020), which aims to define and implement a sustainable model for the scientific and technical cooperation on HTA in Europe. There are 81 partners from EU states involved, including regional HTA agencies such as NICE, HAS and AIFA. An Early Dialogue Working Package (WP5) which involves EUnetHTA and the European Medicines Agency (EMA) has been established to determine how there can be successful collaboration between regulatory bodies and HTA agencies, throughout the life cycle of a technology (see **Figure 1**). There have been other examples of integration over the years, including joint NICE/EMA scientific advice, with the first NICE/FDA joint scientific advice scheduled to happen later this year. The EUnetHTA/EMA collaboration is expected to bring synergies in horizon scanning, scientific advice and the collection of real world evidence.

> **"Collaboration is essential, but all stakeholders must enter the process knowing that they might not get what they want"**
>
> Chris Henshall
> Founding President of HTAi

Figure 1: EUnetHTA in the life cycle of technologies



Adapted from EUnetHTA. REA: Relative Effectiveness Assessment.

Patient Involvement in HTA

The engagement and involvement of patients in HTA processes continued to be a key focus at this year's congress, with 6 panel sessions, 14 oral and 16 vignette presentations dedicated to the topic, as well as one workshop. The key themes of the discussions were:

- Many agreed that education and communication with patients and patient groups still needs to be improved, especially to enable meaningful patient contributions. Progress in this area was demonstrated in three presentations describing the recent updates to the patient expert materials used in NICE technology appraisals.[2,3,4]
- Consideration of ethics was discussed in detail, including the question of which patients should be involved, the need to ensure patient autonomy and independence when involved in HTA processes, and to balance and reconcile public and patient preferences.
- The risk of greater involvement of patient groups leading to more, not less, inequity was touched on, in particular relating to diseases for which no patient organisations exist.
- Rare diseases were mentioned as a unique case where patients are particularly motivated due to serious unmet needs, and parties need to work together across diseases to gain a critical mass for progress.[5]
- The contrast of largely qualitative data from patient experiences and the quantitative data required by decision makers was raised. Particular emphasis was given to incorporating patients at an early stage to ensure drug development focuses on achieving the outcomes most valued by patients.[6,7]

HTA in Low and Middle Income Countries

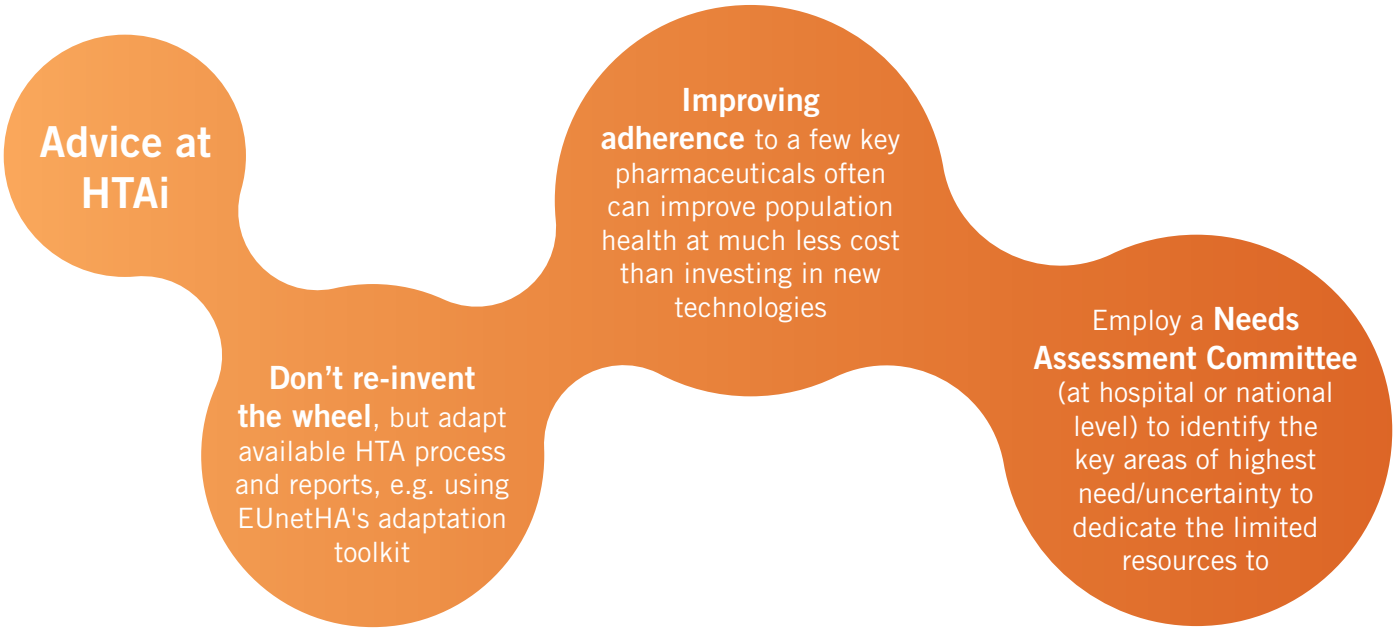
The point was made in the first plenary session that HTA in developing countries is more about the HTA of public health interventions (e.g. drinking water) rather than individual specialised technologies, as it is the public health initiatives that the clinicians can implement and want guidance on.[1]

The following challenges were discussed for developing HTA processes in low and middle income countries in other sessions at the conference:

- Hurdles to establishing frameworks and getting them up and running, alongside minimising duplication and optimising communication
- Difficulty in recruiting educated individuals
- Lack of understanding of HTA from the healthcare community
- Finding funds

The key pieces of advice from speakers at HTAi on how to develop efficient HTA systems in resource limited settings are described in **Figure 2**.[8]

Figure 2: Advice from speakers at HTAi on how to develop HTA processes in resource limited settings



HTA for Orphan Diseases

The challenges associated with the HTA review of high cost drugs for orphan indications was a popular topic with the delegates at HTAi this year. The challenges discussed included the lack of comparator data, the misalignment of the clinical evidence base with the marketing authorisation, the limited trial duration, the lack of patient reported outcome data and uncertainty over cost-effectiveness. The other key issue of whether society was willing to pay more for these drugs, and if so how much more, was also central to discussions. In one panel session, the potential changes to the system in Scotland (for the SMC) and England & Wales (for NICE) were introduced by members of these HTA bodies (see **case studies**).

Case Study – SMC

Potential Changes for the SMC in the Review of Drugs for Very Rare Conditions

Owen Moseley, Senior Health Economist at the SMC

It was indicated at HTAi that the following recommendations from the recent Montgomery report (Dec 2016)[9] would likely to be put into practice:

1. Greater use of conditional acceptance and managed access schemes
2. Changing the definition of orphan, so that it's not only based on patient numbers (it was noted that this new definition would need to be sensitive and specific, and also flexible)
3. Decisions regarding ultra-orphan drugs may be taken outside of the SMC (although it was noted that this could lead to unclear requirements for the evidence needs of the new decision making body)

Case Study – NICE

Potential Changes for NICE in the Review of Drugs for Very Rare Conditions

Sheela Upadhyaya, Director of the Highly Specialised Technology Programme at NICE

The HST Programme at NICE currently have a set of proposed changes to their methods out for consultation. Sheela Upadhyaya, Director of the Highly Specialised Technology Programme at NICE, did not seem convinced that these were the 'right' changes, but she recognised that change was required and welcomed comments on the following proposed changes:

1. Budget impact test, where technologies with a budget impact of >£20m had to enter separate discussions with NHS England around affordability
2. New threshold of £100,000 per QALY (for cases where there are <10 incremental QALYs) but where technologies with an ICER greater than this could still enter the NHS England prioritisation process
3. For cases where there are more than 10 QALYs, the threshold could be raised on a sliding scale according to the magnitude of the clinical benefit. The maximum threshold would be £300,000 per QALY, which could be reached at 30 or more additional QALYs

Despite the greater reliance on QALYs for decision making, it was made clear that the HST committee at NICE would continue to take into account a wide range of 'modifiers' and would be pragmatic around the methodologies used to generate the QALYs from the paucity of evidence, including the use of clinical and patient opinion within the quantitative analysis. Additionally, Sheela was adamant that these reforms would not close the door on managed access agreements, which have proved to be very useful schemes for allowing access with evidence development for these orphan medicines.

Value Frameworks

There was much discussion around value frameworks, and whether or not they add value to the technology. Recently there has been an increase in the number of value frameworks developed by third-party organisations, especially within the US where no national system for technology assessment has been established. It was suggested that the reason for the proliferation of value frameworks may be due to the increased cost of new technologies, a more personalised medicine approach, and the finite resource pool from where these treatments can be funded. However, rather than 'reinventing the wheel' there should be adaptation from more widely established value frameworks. What is meant by 'value' and what could be considered a 'valued health technology' was debated, with the idea of value differing between countries, stakeholders and the context in which it is being considered. Societal value was discussed, including whether or not this should be considered as part of an assessment.

The fifth symposium focused on a different type of value framework, BEACON, which is being used within pharmaceutical companies to ensure that there is internal alignment on the value of a technology.[10] This could be considered more of a 'communication framework' but allows for a clear value message to be established for a technology, as well as the strengths, weaknesses, opportunities and threats surrounding each technology.

Big Data

Big data was a major topic of the meeting with one plenary session, two oral presentation sessions, two vignette presentation sessions and 9 posters on the subject.

The plenary session focussed on mastering big data for effective HTA. With the faster than exponential generation of new data, it was argued that healthcare is becoming a big data science and will need to bridge the gap between biological and clinical medicine

research with the use of universal standards and codes of practice, e.g. the Fast Healthcare Interoperability Resources (FHIR) specification.[11]

Big data can explain both drivers of health outcomes and costs, and is a vital source to measure the performance of health systems by providing insight into the system as a whole, as opposed to the narrow vision of RCTs. The incorporation of RWE into HTA was discussed in depth with successful examples of this coming from Australia's Population Health Research Network (PHRN) which has been able to link administrative data with both health and non-health data as a vital source for HTA and post-marketing analyses, with the further potential of inclusion of genomic and other data in the future.[11]

In some sessions, concerns were raised over whether HTA bodies and publishers value RWE as highly as that from RCTs. In terms of publisher concerns over RWE, a survey had been conducted to assess the views of journal editors on RWE. Overall, there appeared to be a higher threshold for acceptance of RWE studies than RCTs as editors received far fewer RCTs than RWE studies so were more selective of RWE studies.[12]

HTA of Telemedicine

The unique challenges of conducting research and developing economic evaluations for telemedicine initiatives were discussed in one panel session, given the sudden rise in the number of data collection and health management apps that has occurred in recent years.[13]

Key challenges for clinical research that were highlighted:

- The rapid changes in technology, meaning that trials quickly become outdated
- The difficulty of choosing a suitable control
- The problems with ensuring measurement of the same outcomes for each group

Ethical guidelines were also called for to give manufacturers of the technologies a framework for good research and publication practice. There are very few published high quality health economic studies of telemedicine where all costs, including those of the equipment required to employ the telemedicine programme, were included.

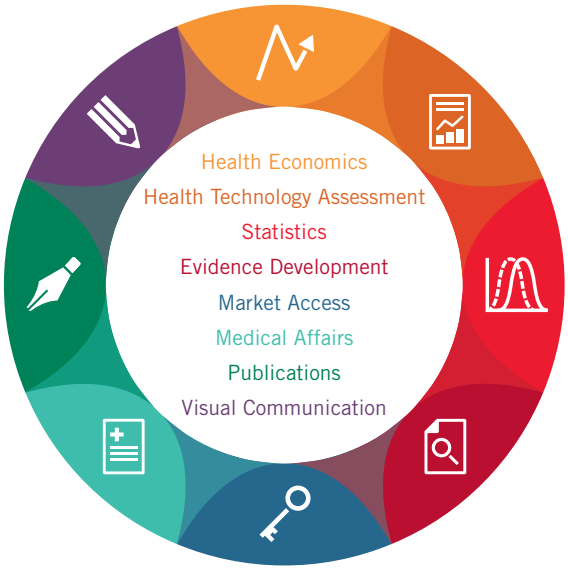
References

1. Plenary Session 1: Towards an Integrated HTA Framework for a More Sustainable Health Care Ecosystem: Principles and the Way Forward
2. VP61 Patient Views of HTA at NICE: Enhancing Involvement Opportunities
3. VP63 NICE Technology Appraisal Patient Expert Feedback: 15-month Analysis
4. OP140 Pictures Speak Louder Than Words: Visuals to Explain NICE Guidance
5. PN17 Rules of Engagement: Motivations for Engaging Patients in HTA Decisions
6. OP10 Myeloma Patient Value Mapping
7. OP121 Social Media to Collect Patient Perspectives on HRQoL in Melanoma
8. PN22 Working on Clinical Practice Guidelines (CPGs) and HTAs in Resource-Limited Settings: Experience from Eurasian Countries
9. http://www.gov.scot/Publications/2016/12/9192/0
10. SY05 Making Drug Development Work: How R&D Can Provide the Answers HTA Assessment Bodies Need
11. Plenary Session 2: Embracing a Value-Based Paradigm in Health Care Systems: Engaging Professionals and Mastering Big Data for Effective HTA
12. VP02 Are Journal Editors a Barrier to Publication of Real-World Evidence?
13. PN04 Methods for Assessing the Value of Technologies: What Have We Learned So Far?

Costello Medical Consulting

Costello Medical provides scientific support to the healthcare industry in the analysis, interpretation and communication of clinical and health economic data. Due to growing demand across an increasing range of service offerings and geographies, Costello Medical has grown organically since foundation in 2008 to a team of over 100 based in Cambridge, London and Singapore.

Alongside our widening technical and creative capabilities, we remain committed to our core values of high quality scientific work coupled with exceptional customer service at competitive and transparent prices. Our talented team has experience with a variety of leading pharmaceutical companies and medical device manufacturers and a track record of success in a broad range of disease areas. For more information on our services in HTA, Health Economics, Statistics, Evidence Development, Market Access, Medical Affairs, Publications or Visual Communication please visit our website at www.costellomedical.com.



Costello Medical will also be attending the ISPOR 20th Annual European Meeting 4th–8th November 2017, Glasgow, UK

Further Assistance

If you would like any further information on the themes or research presented above, please do not hesitate to contact Jeanette Kusel, Scientific Director, at: jeanette.kusel@costellomedical.com.