Where do we Need Good Research Practice Guidance in Health Technology Assessment?

ISPOR 20th Annual European Congress
Glasgow, Scotland
6 November 2017
Workshop 1

Presenters

- **Finn Børlum Kristensen, MD, PhD,** Professor, University of Southern Denmark, Odense, Denmark
- **Mirjana Huić, MD, PhD,** Assistant Director, Department for Development, Research and Health Technology Assessment, Agency for Quality and Accreditation in Health Care and Social Welfare, Zagreb, Croatia
- **Wim Goettsch, PhD,** Director EUnetHTA JA3, EUnetHTA JA3 Directorate, The National Healthcare Institute (ZIN), Diemen, The Netherlands
- **Sophie Werkö, PhD, MSc,** Project Director, Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU), Stockholm, Sweden

Purpose of the Workshop

- To discuss **where good practices have not yet been identified**, and **how the situation could be improved** at European and global scale

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Overview of the Working Group

Title: Overview of Health Technology Assessment (HTA) Approaches to Support Healthcare Decision Making with a Focus on Identifying Good Practices: An ISPOR HTA Council Working Group Report

The **purpose** of the **ISPOR HTA Council Working Group**

To provide an **up-to-date review of current practices with a focus on identifying best practices in the use of evidence to inform health care decision making**
Emphasis was mainly **on approaches to inform population-based purchasing, reimbursement, and formulary decisions on pharmaceuticals, medical devices and other health technologies** while not excluding clinical practice guideline or pathway development

The **rationale** for undertaking this effort

- Identifying good practices in using evidence to inform population-based health care decision making as an **important step forward in capacity building, education, and greater consistency in approaches to HTA-informed decision making**

The **primary audience**

- **Those managing, designing or improving HTA processes** (informative to a wider audience of patients, care providers, payers, academics, and industry stakeholders)

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Two Separate Documents

1) A **background report** with a **summary of key references related to identified good practices in HTA**

2) A **consensus recommendations report** that outlines **where there appears to be best practices** and **where best practices are still emerging or could not be identified** with **a view to prioritizing next steps**

Working Group Members/Authors

- **Finn Børlum Kristensen, MD, PhD,** ***(co-chair)*** Former EUnetHTA Executive Committee Chairman and EUnetHTA Secretariat Director and Professor, Faculty of Health Sciences, University of Southern Denmark, Odense, **Denmark**
- **Don Husereau, MSc, BScPharm**, ***(co-chair)*** Senior Associate, Institute of Health Economics; Adjunct Professor, School of Epidemiology, Public Health and Preventive Medicine, University of Ottawa, Ottawa, **Canada**
- **Federico Augustovski, MD, MS, PhD,** Director, Economic Evaluations and HTA Department, Institute for Clinical Effectiveness and Health Policy (IECS), Buenos Aires, **Argentina**
- **Marc Berger, MD,** New York, NY, **USA**
- **Kenneth Bond, MA, BEd, BA,** Director, Patient Engagement, Ethics and International Affairs, Canadian Agency for Drugs and Technologies in Health (CADTH), Ottawa, **Canada**
- **Andrew Booth, PhD,** Reader in Evidence Based Information Practice and Director of Information, ScHARR, The University of Sheffield, Sheffield, **England, UK**
- **John F. P. Bridges, PhD,** Assistant Professor, Department of Health Policy & Management Johns Hopkins Bloomberg School of Public Health, Baltimore, MD, **USA**
- **Michael Drummond, DPhil, MCom, BSc,** Professor of Health Economics, University of York, York, **England, UK**
- **Jeremy Grimshaw, MBCHB, PHD, FRCGP, FCAHS,** Director, Cochrane Canada and Professor of Medicine,University of Ottawa, Ottawa, **Canada**
- **Mirjana Huić, MD, PhD,** Assistant Director, Agency for Quality and Accreditation in Health Care and Social Welfare, Zagreb, **Croatia**
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- **Daniel Ollendorf, MPH, PhD,** Chief Scientific Officer, Institute for Clinical and Economic Review (ICER), Boston, MA, **USA**
- **Alric Rüther, Dr. med,** Head, International Affairs, Institute for Quality and Efficiency in Health Care (IQWiG), Cologne, **Germany**
- **Uwe Siebert, MD, MPH, MSc, ScD,** Professor of Public Health, Department of Public Health, Medical Decision Making and Health Technology Assessment (HTA), University of Health Sciences, Medical Informatics and Technology (UMIT), Hall in Tirol, **Austria**
- **Jitendar Sharma, PhD,** Director & CEO, AP MedTech Zone & Advisor (Health), Department of Health & Family Welfare, Andhra Pradesh, **India**
- **Allan Wailoo, PhD, MSc, MA,** Professor of Health Economics, ScHARR, University of Sheffield and Director, NICE Decision Support Unit, Sheffield, **England, UK**

Methods

- Followed a **similar approach to that of ISPOR Task Forces**
- **Literature review** and **expert opinion**
- **Reviewed** by all members, revised, shared with a larger review group, and its findings summarized and **presented at ISPOR meetings** (Boston, MA, USA and Glasgow, Scotland)
- Further **revised and circulated to members of the larger review group**
- **Final report**

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Structure

- Reflects a description of components of an HTA process originally developed for the *ISPOR Guidelines Index for Outcomes Research* and enhanced by the *HTA Council Working Group members* based on a **characterization of healthcare decision making** and **relevant components** of an **HTA process:**

Defining the HTA Process - Contextualizing Evidence - Implementing and Monitoring HTA

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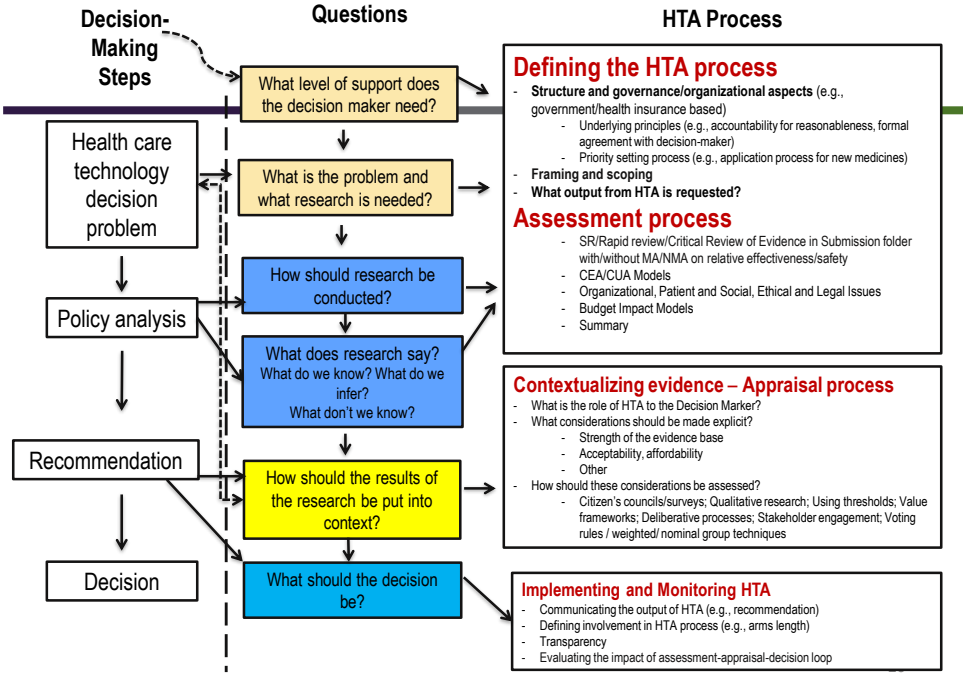
International Society for Pharmacoeconomics and Outcomes Research
Generating Health Care Evidence
Synthesizing Health Care Evidence
Using Evidence in Health Care Decisions
OUTCOMES RESEARCH GUIDELINE INDEX
Guideline Index for Outcomes Research and Use in Health Care Decision Making
GENERATING HEALTH CARE EVIDENCE
SYNTHESIZING HEALTH CARE EVIDENCE
USING EVIDENCE IN HEALTH CARE DECISIONS


Decision-Making Steps
Questions
HTA Process
Health care technology decision problem
Policy analysis
Recommendation
Decision
What level of support does the decision maker need?
What is the problem and what research is needed?
How should research be conducted?
What does research say? What do we know? What do we infer? What don't we know?
How should the results of the research be put into context?
What should the decision be?
Defining the HTA process
- Structure and governance/organizational aspects (e.g., government/health insurance based)
- Underlying principles (e.g., accountability for reasonableness, formal agreement with decision-maker)
- Priority setting process (e.g., application process for new medicines)
- Framing and scoping
- What output from HTA is requested?
Assessment process
- SR/Rapid review/Critical Review of Evidence in Submission folder with/without MA/NMA on relative effectiveness/safety
- CEA/CUA Models
- Organizational, Patient and Social, Ethical and Legal Issues
- Budget Impact Models
- Summary
Contextualizing evidence – Appraisal process
- What is the role of HTA to the Decision Marker?
- What considerations should be made explicit?
- Strength of the evidence base
- Acceptability, affordability
- Other
- How should these considerations be assessed?
- Citizen's councils/surveys; Qualitative research; Using thresholds; Value frameworks; Deliberative processes; Stakeholder engagement; Voting rules / weighted/ nominal group techniques
Implementing and Monitoring HTA
- Communicating the output of HTA (e.g., recommendation)
- Defining involvement in HTA process (e.g., arms length)
- Transparency
- Evaluating the impact of assessment-appraisal-decision loop

Manuscript Sections

- HTA Terminology
- Framework / Principles For HTA Processes
  - Structure / Governance / Organizational Aspects Of HTA
  - Priority setting for HTA
  - Framing and scoping research
- Synthesizing Evidence
  - Overview of issues related to conduct and reporting of clinical and economic evidence
  - Best practices in interpretation of individual studies
- Using Evidence
  - Equity issues and economic evaluations
  - Ethics
  - Integrating stakeholder input (e.g., patients, clinicians) and considering social values to support decision making
- Implementing HTA
  - What should be transferred?
  - To whom should HTA results be transferred?
  - By whom should HTA results be transferred?
  - How should HTA results be transferred?
  - Implementation strategy - reimbursement and pricing of drugs
- Measuring HTA Impact
- The Future Of HTA

Defining the HTA Process

Structure / Governance / Organizational Aspects Of HTA

- **There are several proposed governance models** and **governance indicators** for healthcare systems in both developed and less developed systems that may intuitively be applied to HTA processes

Framework / Principles For HTA Processes / Interpreting Research

- **Key known principles for the conduct of HTA**
- **Principles to guide and benchmark HTA organizations**, particularly those in low- and middle-income countries, **may be difficult to achieve**, either through lack of funding or local institutional barriers

Using Evidence (Appraisal Process)

- **Contextualizing the evidence** for a particular jurisdiction along with incorporating additional social values through considering stakeholder input, and supporting the implementation of decisions
- **Transparency** of the appraisal process can **be improved** by using an **explicit decision framework**
- Systematic use of such a framework enhances consistency across decisions, allows justification of value judgments, and thus enhances legitimacy of societal decision making

Implementing and Monitoring HTA

- A **plan to maximize the likely impact** of the HTA should be developed
- A **robust approach** that requires a broad range of research methods is **still needed**
- Published **evidence on the HTA impact in different jurisdictions**

Viewpoints

What areas of HTA are in need of guidance and good research practice documents and **how should we address them?**

EUnetHTA and **national HTA institution**	**INAHTA** and **national HTA institution**
Wim Goettsch, PhD, Director EUnetHTA JA3, EUnetHTA JA3 Directorate, The National Healthcare Institute (**ZIN**), Diemen, The **Netherlands**	**Sophie Werkö**, PhD, MSc, Project Director, Swedish Agency for Health Technology Assessment and Assessment of Social Services (**SBU**), Stockholm, **Sweden**

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ISPOR Good Research Practices in HTA (GPA) and EUnetHTA

A focus on the assessment phase

Wim Goettsch

Director EUnetHTA JA3 Directorate

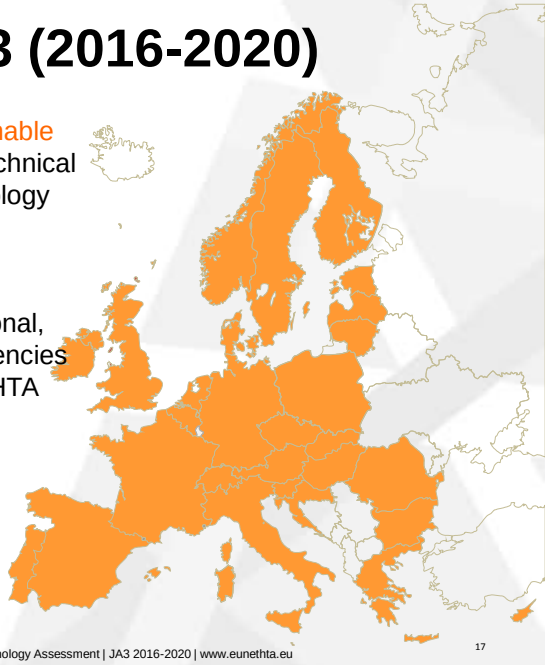
ISPOR Glasgow, November 6, 2017

eunethta

European Commission

EUnetHTA JA3 (2016-2020)

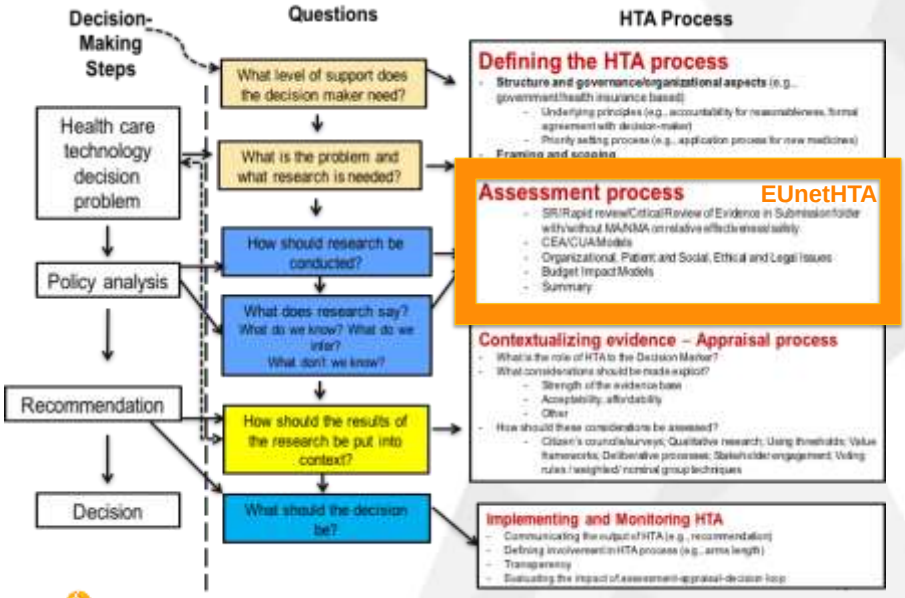
Aims to contribute to a sustainable model for the scientific and technical cooperation on Health Technology Assessment (HTA) in Europe



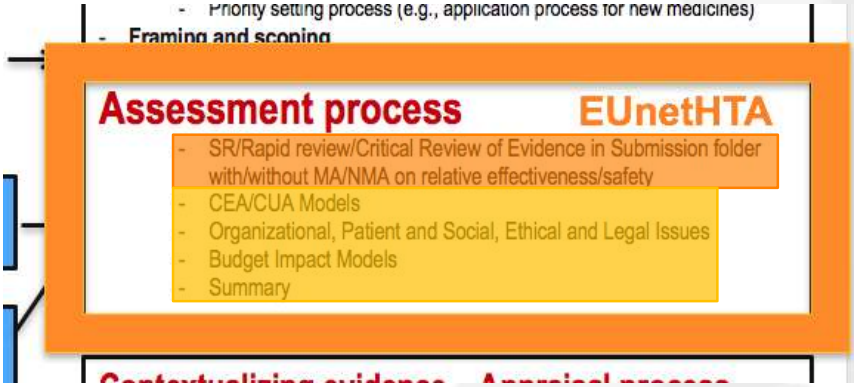
81 partners consisting of national, regional and non-for-profit agencies that produce or contribute to HTA

Project Coordinator:
Dutch National Health Care Institute (ZIN)

Fit EUnetHTA activities to the GPA scheme?

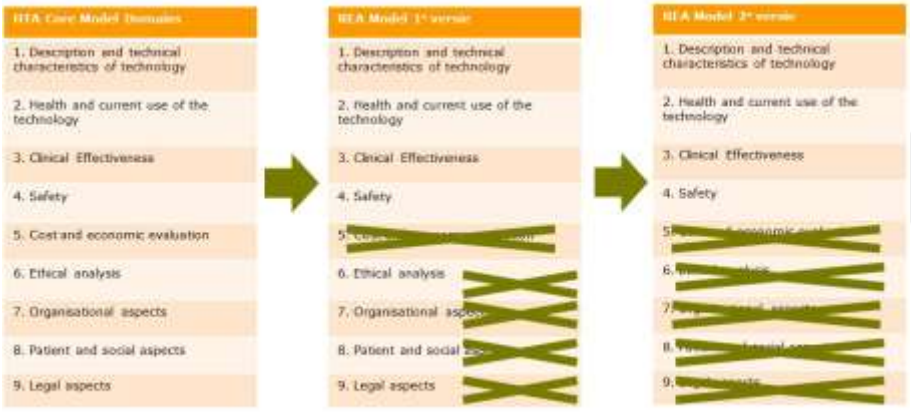


Topics to be further worked out by EUnetHTA?



- Differents levels:
  - International assessments will be mainly focussed on clinical assessments;
  - National assessments will also have focus on non-clinical domains.

Selection of the clinical elements for the joint reports



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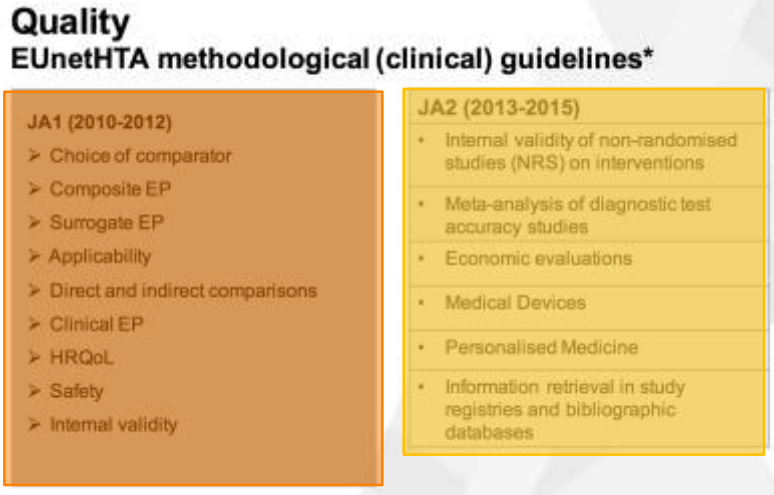
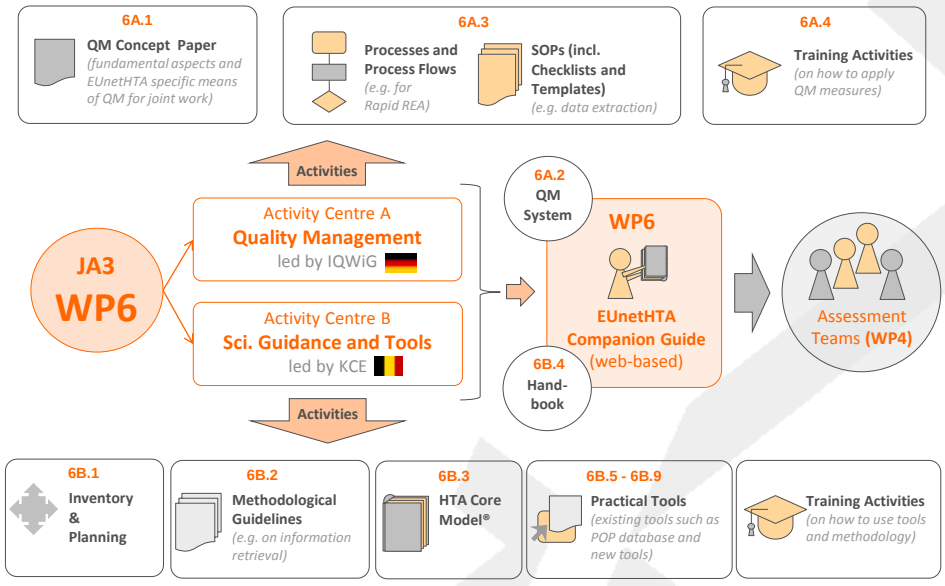


Topics to be developed further as part of GPA relevant to EUnetHTA

- **Direct and indirect comparisons**
  - Network Meta Analysis (NMA) (ISPOR Task Force 2014);
- **Clinical, surrogate and composite endpoints (incl. QoL)**
  - Bringing relevant endpoints together for different therapeutic indications (oncology) --- not only HTA but also EMA, clinicians etc;
- **Economic analysis**
  - Economic models (Guideline EUnetHTA, CHEERS Statement 2013)
  - Budget impact (ISPOR Task Force 2014);
- **Organizational, Patient and Social, Ethical and Legal Issues**
  - HTA core model, INTEGRATE, etc.

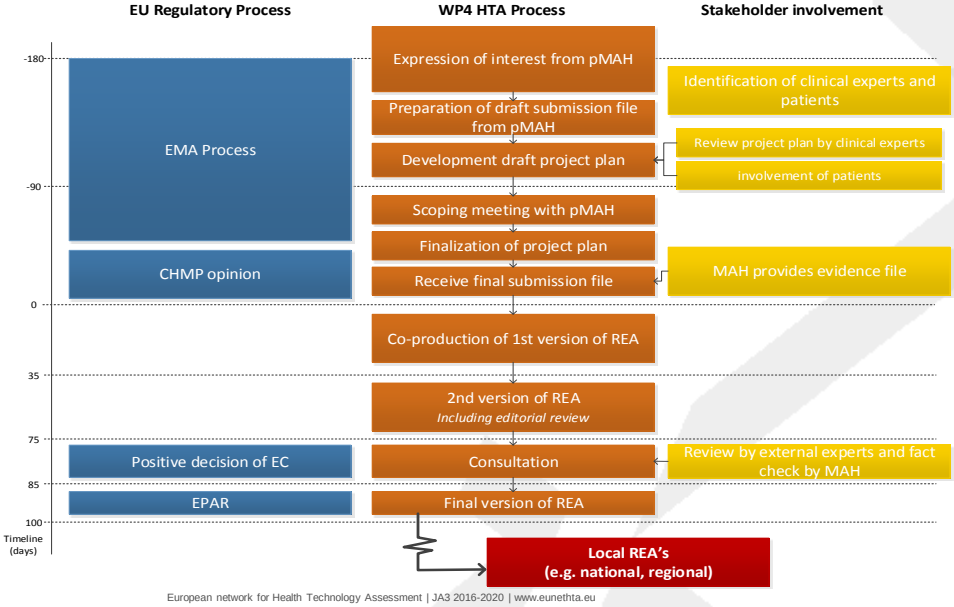
What about the context of assessment processes (I)?

WP6 – Organisation of work



What about the context of assessment processes (II)?

Interaction with EMA and stakeholders

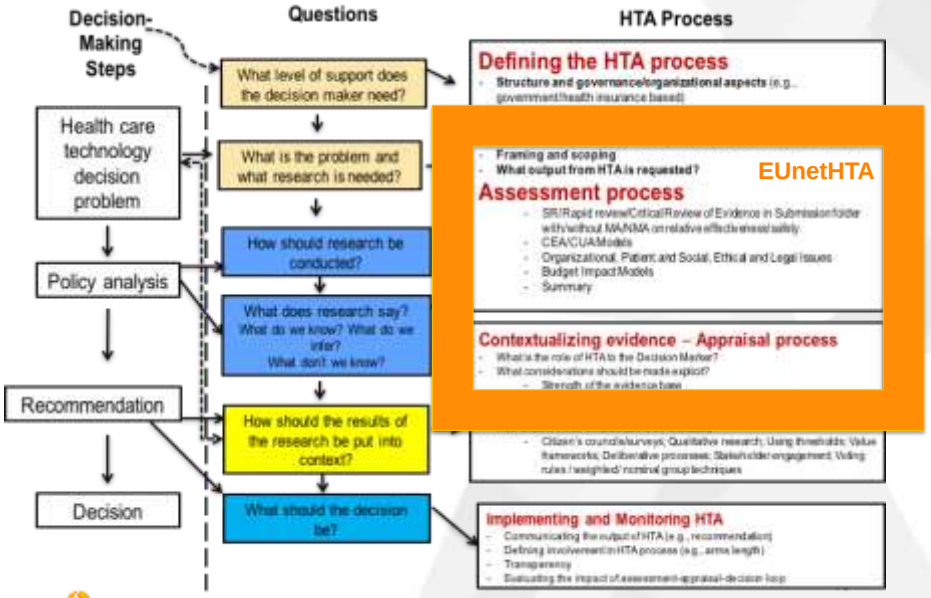


Conclusions

- For European collaborations such as EUnetHTA the focus with *Good Research Practices* seems to be mostly on *Assessment process* with the total *HTA process*
  - It is important to differentiate in this process between activities that support international collaboration in assessments (Joint REAs) and activities that support national, regional or even local assessments;
  - In the clinical domain, alignment is most likely but sometimes difficult in expanded network of organisations within HTA (ISPOR, HTAi, EUnetHTA, etc.) and outside the HTA domain (Cochrane, EMA, healthcare providers);
  - Outside the clinical domain more collaboration is also possible but is sometimes hampered by political considerations.

- The EUnetHTA assessment process should not only be dedicated to methods but should also include the overarching processes within but also outside HTA
  - Parts of the Good Research Practices such as *framing and scoping* and *contextualizing the evidence,* are also very relevant for EUnetHTA.

Evolve EUnetHTA activities to the GPA scheme?



SWEDISH AGENCY FOR HEALTH TECHNOLOGY ASSESSMENT AND ASSESSMENT OF SOCIAL SERVICES

Sophie Werkö,
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ISPOR Glasgow, November 6, 2017

INTRODUCTION TO INAHTA

INTERNATIONAL NETWORK OF AGENCIES FOR HEALTH TECHNOLOGY ASSESSMENT

INAHTA MEMBERS

- 50 agencies from 31 countries:
  - 40 in High income countries
  - 8 in Upper-middle income countries
  - 2 in Lower-middle income countries

- Agencies by region:
  - 28 Europe
  - 6 Latin American countries
  - 3 Australia & New Zealand
  - 5 Canada & USA
  - 6 Asia
  - 2 Africa

ROLE OF INAHTA

- A network of HTA agencies
- All member agencies:
  - are publicly funded and not-for-profit
  - assess health technologies to support national or regional health system decision making
- Provides a platform for member agencies to share knowledge and learn from each other
- Has partner relationships with WHO, HTAi, HTAsiaLink, and many others
- Questions? Visit the INAHTA website for contact information: www.inahta.org

4 PRINCIPLES RELEVANT TO ALL HTA AGENCIES

- Relevance
- Quality
- Timeliness
- Impact

Means that for the ISPOR paper to be relevant for agencies, it needs to be practical, feasible, implementable and cost-effective.

WHAT AREAS OF HTA ARE IN NEED OF GUIDANCE AND GOOD RESEARCH PRACTICE DOCUMENTS AND HOW SHOULD WE ADDRESS THEM?

We need guidance on:

- What kinds of deliberative practices are most effective?
- How to ensure that stakeholder engagement is meaningful?
- How to incorporate a lower level of evidence into our HTA practices, if we should? We require good practices for conducting reassessments based on observational data, real world data, etc.

FURTHER, WE ALSO NEED:

- Additional research on assessing impact
- Research on how HTA leads to behaviour change amongst clinicians
- Guidance on adaptation of HTA reports across jurisdictions
- A much greater focus on supporting implementation - how do we go beyond cost-effectiveness to address the important issue of affordability?
- Adaptation of HTA to meet new challenges

HTA REQUIRES AN INCREASED APPLICATION OF OTHER FACTORS

- Alignment with regulators
- Ethical, legal, and social issues
- Environmental concerns
- Implementation considerations contextualized to the region in question

HTA NEEDS TO ADDRESS PAYER CONCERNS ABOUT AFFORDABILITY

Questions for the Audience

- What are the **further areas in need** of guidance and good research practice documents?
- What are the **suggested approaches of how to address them?**
- **How the situation could be improved** at European and global scale?
- How can this document **help with global alignment of HTA?**

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