HTx – 3rd Forum Meeting
June 7, 2021

HTx Forum in 2019

HTx 2020 started normally......

But it changed quite dramatically......
Challenges 2020-2021

• Face-to-face meetings not possible anymore
  • Contributing to delays in deliverables
  • Were finally replaced by virtual meetings
  • But still missing the informal contacts between internal and external partners
  • Data exchange became an even bigger issue
  • Slower processes to obtain data access
  • No possibilities for people to move around in order to get access
  • Also remote working was more difficult, for instance installing data servers

Possibilities

• Much easier to link up and organise meetings
• Involving experts from all over the world
• More efficient use of working time, but…
• To get a better perspective on each other homes;)
• Find different ways to interact.

Some highlights from the work in HTx

- The HTx video (link)
- A lot of external activities in terms webinars, workshops, focus groups
  - HTx Pre-workshop Transferability (WP5, Syreon)
  - Workshop Technologies for COVID-19 (WP4, NICE)
  - HTx Webinar Case study 1 Proton Therapy (WP1, EORTC, UMCG, UMCU)
  - Focus groups interaction regulators, HTA, clinicians (WP4, UU)
  - Presentations (33), posters (11) and slide-decks (7) at conferences such as ISPOR & HTAi but also more ‘technical conferences such as statistic, diabetes and AI
  - Peer reviewed publications (5)

Conversation with the Forum members 1/2

In what way are the aims of HTx (using RWD, personalized medicine, prognostic models, patient centricity) part of the conversations you are having at your organisation? In what way are they discussed? With caution/excitement?

What are the issues you are struggling with when collecting and analysing all the data you need? Can the case studies provide examples for dealing with these struggles? In what way?

Is transparency and building trust for your methods an issue at your organisation? How are you trying to achieve this?

Are you convinced that the outcomes of the case studies will be a good example of how new methodologies can be developed using RWD? Why or why not?
What would convince pharma companies to share more RCT-data?
What would convince patients to take part in research or make their data available?

A Systematic Review of Collective Evidence Investigating the Effect of Diabetes Monitoring Systems and Their Application in Health Care

Process on how to innovate HTA methods
Developments in the coming year

- More results coming available from the methods WPs and CS
- Continuous focus on relevance of those methods for HTA organisations
- More involvement external stakeholders in HTx projects
- Increased collaboration with other international projects such as IMI-EHDEN
- Hopefully also more possibilities to physically interact
- Link to the HTAi meeting in 2022 in the Netherlands

Identified challenges of using RWD in HTA

Based on a study of present RWD-use in 25 EU HTA-authorities:

"Overview of the development of the use of RWD including a review of international consensus methods currently developed." HTx deliverable 4.4

Johan Pontén, Senior Advisor International Affairs, TLV

Survey on use of RWD in HTA-authorities

- Survey on the use of RWD
- 67% (24/36)
  - 15 questions on RWD use
  - Basis for selection for interviews
- 9 (10) Interviews
  - Thematic overview of the answers
- Literature review
- European initiatives for methods development for RWD use
Resolution of uncertainties in determination of value

Initial HTA
- describe current standard of care
- create external comparator to contextualize efficacy
- populate cost effectiveness and budget impact models

Managed entry agreement
- evaluate outcomes in clinical practice
- resolve uncertainties related to determination of value in the initial assessment

Re-assessment
- complement the clinical and economic evidence base that was available in the initial assessment
- monitor utilization and evaluate budget impact in clinical practice

Facey et al., 2020, Real-world evidence to support Payer/HTA decisions about highly innovative technologies in the EU—actions for stakeholders. International Journal of Technology Assessment in Health Care 1–10.

Potential use of RWD

<table>
<thead>
<tr>
<th>Reason for not using RWD – 1st rank</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rank the following issues of reasons for not using RWD from top (most important reason) down (least important or no reason at all) Rank 1</td>
</tr>
</tbody>
</table>
| 1. Lack of evidence of effectiveness/efficiency regarding drug use costs compensating or qualifying out of pocket
| 2. Lack of evidence of effectiveness/efficiency regarding drug use costs compensating or qualifying out of pocket
| 3. Lack of evidence of effectiveness/efficiency regarding drug use costs compensating or qualifying out of pocket
| 4. Lack of evidence of effectiveness/efficiency regarding drug use costs compensating or qualifying out of pocket
| 5. Lack of evidence of effectiveness/efficiency regarding drug use costs compensating or qualifying out of pocket

Reasons for not using RWD – 1st rank

Current situation in HTA-authorities

- We see that many agencies (only) have access to claims data – some important questions can be answered by that, but not all
- Data on effectiveness is difficult to get
- Proxy data can be used for some effects
- Indication is a difficult variable to catch in many countries
- Effectiveness and resources use in clinical praxis are contexts where RWD is very interesting
Data access and processing

• Many agencies are working to improve data access
• Legal frameworks for data access exist in some countries, but others are trying to put them in place.
• Infrastructure for gathering of data can often become a question about the burden on health care staff for additional filing
• Register study competencies can be found inhouse at some agencies but also competencies at registry holders are used

Theoretical Framework

Policy factors relevant to build the use of RWD

Policy Conclusions

• Policy is ranked in the survey as prime hindrance for the use of RWD
• Leadership and Vision is primordial – other factors can be changed with time
• Few autorities have refied knowledge that is a support for RWD processes (contracts, processes, guidelines and so on)
• Individuals can drive change – but not build a system if leadership is not there.
• Build networks to learn from others!
• In-house competence can be a key to success
• Access to data, low impact on the health care staff, legal support are other success-factors
Challenges in HTA of ‘complex’ technologies

Before developing new methods in HTx
What exactly are the current challenges that require updated methods?

Our aim
To which extent do European HTA organisations perceive HTAs of complex therapies as challenging and what are the main challenges are in these HTAs?

Survey to European HTA organisations

Identify which health technologies are considered complex
Identify challenges encountered in HTA of complex technologies

Research question
Sub-questions

Specifying specificity

Gap analysis of challenging HTAs

Validity of specific health technologies

identifying specific health technologies

Part 2 - Case studies

- What were the main challenges encountered in the case study?
- What was the health technology involved?

Part 3 - Openness

- What were the main challenges encountered in the case study?
- What was the health technology involved?

Multiple choice
Open
Contribution HTA organisations

22 organisations from 21 countries completed the questionnaire!

- Mix of Eastern & Western European countries
- Most assess pharmaceuticals (95%); some medical technologies (40%)

29

Summary of description why HTA was challenging

30

31

32
Describe challenges

2/3 arguments is data related

• First expressed during relative effectiveness assessment (REA)
• Result in uncertain input for cost-effectiveness assessment (CEA) and decision making

Most relate to immature data or ‘limited’ data
• Large share contributed to Quality of Life data

To conclude

Challenges in HTAs of complex health technologies mainly root in data insufficiencies

• Results in outcome uncertainties during the REA
• In parameter uncertainty in the CEA
• Ultimately complicates decision making

Solutions
1. Improve data quality and quantity
2. Evidence-synthesis methods able to deal with data insufficiencies
3. Pricing and reimbursement schemes that mitigate risks

THANK YOU!

Thanks to others working in the HTx Project:
- Prof. Aukje K. Mantel-Teeuwisse
- Dr. Wim G. Goettsch
- Rick A. Vreman, MSc, PharmD

Thanks to the collaborating institutes:
- University of Copenhagen
- ZIN
- NICE
- TLV