HTx Focus Group

Synergies between regulatory authorities, HTA organisations and clinical guideline developers

May 10th 2021
General remarks

- This session will be recorded
- Please mute yourself when not speaking
- For short questions use the chat
Content

- Background of HTx
- Today’s focus
- Why you are here
- Four case studies
- Today’s schedule
- Moderators
Presenters

- **Dr. ir. Wim Goettsch**
  - Special advisor HTA at the Dutch National Health Care Institute
  - Associate professor HTA at Utrecht University, PI of the HTx project

- **Milou Hogervorst, PharmD, MSc**
  - PhD candidate in HTx at Utrecht University
Background HTx

- Horizon 2020 project supported by the European Union, kicking-off in January 2019 and lasting for 5 years.

- Facilitate the development of methodologies to deliver more customized information on the effectiveness and cost-effectiveness of complex and personalised combinations of health technologies.

- Provide methods to support personalised treatment advice that will be shared with patients and their physicians.

- In close collaboration with the European Network for HTA (EUnetHTA) and its stakeholders pilot the implementation of these methods in Europe.
The HTx project participants?

- Utrecht University (project coordinator) (UU) Netherlands
- University of Copenhagen (UoC), Denmark
- University of Oulu (UoO) Finland
- University of York (UoY) UK
- Medical University of Sofia (MUS) Bulgaria
- University of Bern (UBERN) Switzerland
- Universidad Politecnia de Madrid (UPM) Spain
- European Organisation for Research and Treatment of Cancer (EORTC) Belgium
- Dental and Pharmaceutical Benefits Agency (TLV) Sweden
- National Health Care Institute (ZIN) Netherlands
- National Institute of Health and Care Excellence (NICE) UK
- Syreon Research Institute (SRI) Hungary
- Synapse research management (SYNAPSE) Spain
- EURORDIS Rare Diseases Europe (EURORDIS) France
- University of Maastricht (UM) Netherlands
Statistics and artificial intelligence

RWD for evidence synthesis to support decision making
- Statistical prognostic and evidence synthesis methods
- Combining study designs
- Multiple treatment comparisons
- Individualised decision making

AI for predicting treatment outcomes based on RWD
- Machine learning systems
- Combining data sources
- Treatment pathways and sequences
- Individual treatment outcomes

Using 4 case studies
*Head and neck cancer, diabetes mellitus, multiple sclerosis and myelodysplastic syndromes*
## Implementation as key theme

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<th>EUnetHTA letter to DG research in 2015, research focus:</th>
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A changing landscape
Personalisation requires alignment

**Patient population**

“One size fits all” approach to medicine

“personalized” ("precision") medicine

**Treatment decision**

- A is the best treatment on average.
- For some patients A might not be effective and/or safe

- treatment A
- treatment B
- treatment C
- treatment D
Streamlining the process

Current situation

1 Market approval
2 HTA reimbursement
3 Uptake clinical guideline

R&D + RCT → PATIENT ACCESS

Target situation

1 MA
2 HTA
3 CG

Potential for alignment
Our HTx research

R&D + RCT → PATIENT ACCESS

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Ways towards synergy in literature

The need to align evidentiary requirements among stakeholders is an overarching theme across literature

Aligning evidentiary requirements

All respondents support synergy between regulatory and HTA stakeholders

Where could alignment occur?

(A) Acceptable primary end point.
(B) Inclusion of active comparator arm in the trial.
(C) Use of patient reported outcomes.
(D) Use of health-related quality of life measures.
(E) Choice and use of surrogate measures.
(F) Criteria considered in choice of comparator: therapeutic.
(G) Use of subgroup analyses.
(H) Inclusion and choice of secondary efficacy parameters.
(I) Definition of unmet medical need.
(J) Use of biomarkers to monitor patient outcomes. HTA, health technology assessment.
EMA/HTA parallel scientific advice

How aligned are the perspectives of EU regulators and HTA bodies?
A comparative analysis of regulatory-HTA parallel scientific advice
Synergy HTA - guidelines

HTA  59% does not refer to CGs
     57% does not report consultations

CG   2/7 does not refer to HTA reports
     5/7 does not report consultations

Final recommendation (yes/no)
90% identical
Recommended patient population
51% identical

Total no. of comparisons
N = 51

Treatment line unclear
N = 2

Included no. of comparisons
N = 49
- UK: N = 21
- FI: N = 10
- DE: N = 3
- NL: N = 7
- PL: N = 8

Similar
N = 29 (59%)
- Same treatment line
  N = 25 (51%)
- Same treatment line
  start, continues in further lines
  N = 4 (8%)

Minor differences
N = 15 (30%)
- Same treatment line,
  described for more indications
  N = 5 (10%)
- Same treatment line,
  different indication
  N = 5 (10%)
- Treatment starts at different line
  N = 6 (12%)

Major differences
N = 8 (16%)

Hogervorst et al. HTx task 4.1 Synergies between HTA and clinical guidelines (not yet published)
Time from MA application to reimbursement

Figure 3: Average time to availability in days (2015–2018)

Source: EFPIA; EPAR refers to European public assessment report

Source: IQVIA

EFPIA 2020. The root cause of unavailability and delay to innovative medicines: Reducing the time before patients have access to innovative medicines
HTA review time + time lag MA - HTA

Gap MA – HTA submission:
7-42 days

Companies seek advice before submission:
23-73% of total submissions

## Time lag HTA/guideline

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## Current initiatives

### Regulatory - HTA
- EMA/HTA Scientific advice
- EMA/EUNetHTA parallel consultation
- PRIME
- MHRA/NICE Scientific advice programme
- ZIN/MEB parallel review
- MPA/TLV scientific advice
- Tapestry Network pilots scientific advice
- TGA/PBS scientific advice
- TGA/PBAC parallel submission
- Health Canada/CADTH parallel submission
- FDA/CMS parallel submission
- Green park collaborative scientific advice
- ...

### HTA – clinical guidelines
- GINATHA working group
- European Reference Networks
- Magic project
- NICE guidelines
- HAS guidelines
- ...

Komen et al. 2016. Effects of policy interventions on the introduction of novel oral anticoagulants in Stockholm: an interrupted time series analysis
Clinical guidelines to facilitate patient access

No ‘hard’ yes or no, though a large effect

Komen et al. 2016. Effects of policy interventions on the introduction of novel oral anticoagulants in Stockholm: an interrupted time series analysis
Today’s focus

Find tangible ways to improve synergies between the processes of regulatory authorities, HTA organisations and clinical guidelines.

1. To which extent can we converge evidentiary needs among stakeholders?

2. How can we achieve convergence of evidentiary needs among stakeholders?
Topic 1 - Can we converge?

What are the crucial and feasible assessment criteria to align among regulatory authorities, HTA organisations, and clinical guideline developers (according to the PICOT framework)?

- How to define relevant patient populations and subgroup analysis?
- How to agree on characteristics of the intervention?
- How to determine the rightful comparator?
- How to decide on acceptable outcomes?
- How to determine the appropriate trial design?
Topic 2 (1) – How?

How can we employ methods to achieve convergence among stakeholders?
- Which methods are or can be used in the stakeholders' tasks?
- If you would work through similar methods, what would you win and what would you lose?

How can we use early stakeholder dialogue to achieve convergence?
- When in the process should these conversation(s) take place?
- Who should be involved in these conversations?
- Which topics are most relevant to discuss here? (relates to topic 1)
- Who should initiate or lead these conversations?
- What would potentially prevent you from engaging in stakeholder dialogues?
Topic 2 (2) – How?

Are there other potential ways to converge evidentiary needs among stakeholders?

To which extent can we cooperate to achieve convergence?
- Should convergence be about information sharing or actual work load sharing?
- If you would share information or cooperate, what would you win and what would you lose?

How can we guarantee independency of stakeholders while converging?
The four case studies

- Head and Neck Cancer
- Diabetes Mellitus Type 1 & 2
- Multiple Sclerosis
- Myelodysplastic Syndromes
1. Head & Neck Cancer

Use of proton therapy

- Highly expensive
- Effective in specific population

GOAL

Statistical models that facilitate stratified medicine decisions by predicting for which patients’ proton therapy is most beneficial
2. Diabetes Mellitus Type 1 & 2

Combinations of (e-)health technologies

- Traditional medication (insulin + oral treatments)
- Insulin pumps, continuous glucose monitoring, glucose meters, tele-monitoring with data visualisation, life-style interventions

GOAL

Provide individualized treatment and monitoring strategies in patients with different types of diabetes and in different age groups
2. Diabetes Mellitus Type 1 & 2

3. Multiple Sclerosis

Optimal treatment for relapsing-remitting MS

- Many expensive immunomodulating treatments in short period
- The more effective, the more serious adverse events

GOAL
Combine RCT and RWD to estimate treatment effects in subgroups for individualised treatment decision-making
3. Multiple Sclerosis

Use of MS medication in daily practice in the Netherlands
4. Myelodysplastic Syndromes

Comparing treatments for a rare disease

- Hard to diagnose and manage
- Finding optimal treatments based on small populations

GOAL

Developing prediction models and evaluating (cost-)effectiveness of treatment sequences and combinations for individualised treatment decisions using relevant patient reported outcomes (PROMS)
4. Myelodysplastic Syndromes

# Schedule

<table>
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<th>Time</th>
<th>Session</th>
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| 9:30 – 10:00 | Plenary opening (30 mins)  
*Welcome, introduction (organisers) and instruction* |
| 10:00 – 11:00 | Focus group topic 1 (60 mins)  
*To which extent can we converge evidentiary needs among stakeholders?*  
**H&N cancer**  
Prof. dr. Bert Leufkens  
Dr. Rick Vreman  
**DM**  
Prof. dr. Marieke de Bruin  
Dr. Mathias Møllebæk  
**MS**  
Dr. Wim Goettsch  
Milou Hogervorst, PharmD, MSc  
**MDS**  
Prof. dr. Aukje Mantel-Teeuwisse  
Dr. Junfeng Wang |
| 11:00 – 11:15 | Plenary sharing of findings + energizer (15 mins) |
| 11:15 – 11:30 | BREAK (15 min) |
| 11:30 – 12:30 | Focus group topic 2 (60 mins)  
*How can we achieve convergence of evidentiary needs among stakeholders?*  
**H&N cancer**  
Prof. dr. Bert Leufkens  
Dr. Rick Vreman  
**DM**  
Prof. dr. Marieke de Bruin  
Dr. Mathias Møllebæk  
**MS**  
Dr. Wim Goettsch  
Milou Hogervorst, PharmD, MSc  
**MDS**  
Prof. dr. Aukje Mantel-Teeuwisse  
Dr. Junfeng Wang |
| 12:30 – 12:45 | Plenary sharing of findings (15 mins) |
| 12:45 – 13:00 | Closure of session (15 mins)  
*Rankings with mentimeter* |
Our wonderful assistance

- Estefanía Collado
  Synapse research management partners
- Ayla Lokhorst
  Project Manager HTx at ZIN
Moderators Focus Group 1
Head and Neck Cancer

Dr. Rick Vreman
Assistant professor at Utrecht University, research focus on link between drug regulation and HTA
Advisor at the Dutch National Health Care Institute (ZIN)

Ting-An Lu, BSc
Master student Drug Innovation at Utrecht University, working on HTx synergy project as
Moderators Focus Group 2
Diabetes Mellitus

| Prof. Dr. Marieke de Bruin | Professor in Drug Regulatory Science at Utrecht University
<table>
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<th>Previously employed at the EMA (PRAC) and the Dutch Medicines Evaluation Board (MEB)</th>
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<tbody>
<tr>
<td>Dr. Mathias Møllebæk</td>
<td>Postdoctoral Fellow at the University of Copenhagen Centre for Regulatory Science, research focus on medical information artifacts that address regulatory and clinical publics, such as medicine risk advisories and clinical guidelines</td>
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Moderators Focus Group 3
Multiple Sclerosis

Dr. Ir. Wim Goettsch
Special advisor HTA at the Dutch National Health Care Institute (ZIN)
Associate professor HTA at Utrecht University, PI of the HTx project
Former project leader and director at EUNetHTA Joint Action projects

Milou Hogervorst, MSc
PhD candidate in HTx at Utrecht University, research focus on HTA policies and the link between HTA and clinical guidelines
Moderators Focus Group 4
Myelodysplastic Syndromes

Prof. Dr. Aukje Mantel-Teeuwisse
Professor of Pharmacy and Global Health at Utrecht University
Managing Director of the Utrecht Centre of Pharmaceutical Policy and Regulation

Dr. Junfeng Wang
Assistant professor in HTx at Utrecht University, research focus on methods and statistics
Break-Out Focus Group 1

To which extent can we converge evidentiary needs among stakeholders?

60 minutes (10:00-11:00h)
Note

General
- This session will be recorded
- This session will last for 60 minutes, moderators track time

Communication
- Please turn your camera on
- Please mute yourself when not speaking
- For short questions use the chat
Your most important findings (1)

To which extent can we converge evidentiary needs among stakeholders?

H&N?
DM?
MS?
MDS?
BREAK

15 minutes (11:15 – 11:30h)
Break-Out Focus Group 2

How can we achieve convergence of evidentiary needs among stakeholders?

60 minutes (11:30-12:30h)
Note

General
- This session will be recorded
- This session will last for 60 minutes, moderators track time

Communication
- Please turn your camera on
- Please mute yourself when not speaking
- For short questions use the chat
Your most important findings (2)

How can we achieve convergence of evidentiary needs among stakeholders?

H&N?
DM?
MS?
MDS?
Wrap up
HTx Focus Group

Synergies between regulatory authorities, HTA organisations and clinical guideline developers

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Please, go to menti.com

The voting code: 2543 7068
Or use the QR-code:
Take-aways

1. To which extent can we converge evidentiary needs among stakeholders?

2. How can we achieve convergence of evidentiary needs among stakeholders?
Thank you for your participation!

We will update you with a summary of the results this June/July

For follow-up questions or remarks contact Milou Hogervorst: M.A.Hogervorst@uu.nl