



Real-World Evidence for Decision-Making in Healthcare:

HTA Perspectives on Access & Implementation

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| Van goede zorg verzekerd |



Why explore Real-World Evidence (RWE)?

- RCTs: a **golden** standard?
- The efficacy-effectiveness gap (Eichler et al.)
- Expansion of mandates:
 - Systematic evaluation of all aspects of an intervention
 - Questions go beyond treatment X vs. Y (or placebo)
 - Societal perspective of analyses
 - **Different** evidence is also needed

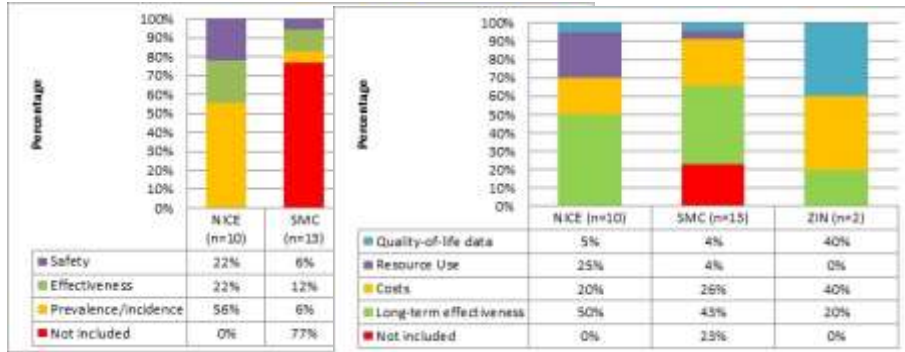




RWE use in HTA Practice: 5 European HTA Agencies

Relative Effectiveness Assessments (REAs)

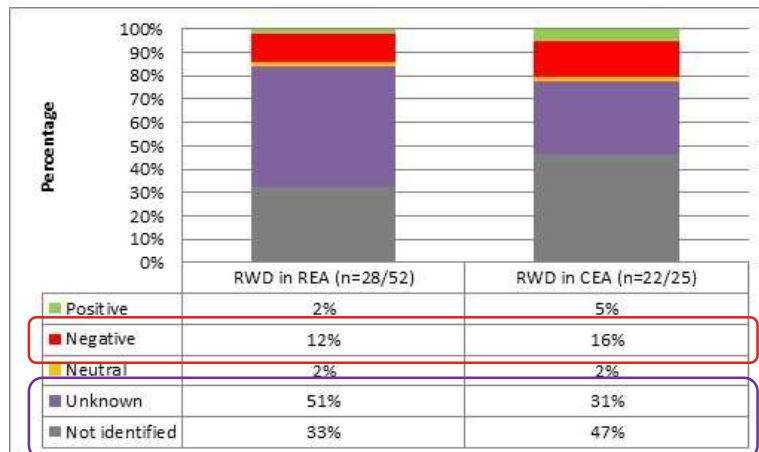
Cost-effectiveness Assessments (CEAs)



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RWE use in HTA Practice: Appraisal of RWE



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RWE in Practice: Conditional Financing

T=0: Assessment

- Therapeutic value & 1e CE/BIA
- Uncertainties identified
- Outcomes research proposal discussed

Outcomes Research

- Dutch clinical practice
- Eg. registries

T=4: Re-assessment

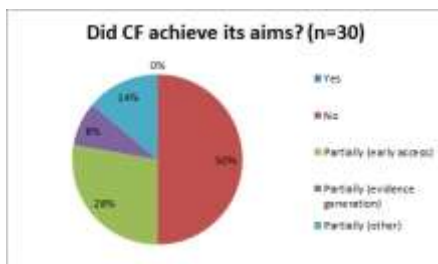
- Conclusions
- Advice to MoH on reimbursement

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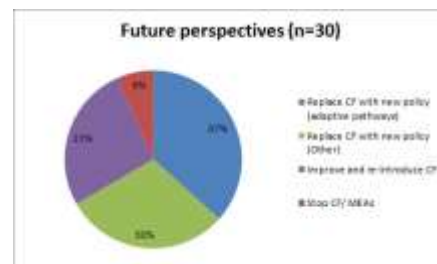


Stakeholder Perspectives (n=30)

Did it work?



Now what?



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Practicalities of RWE use in CER

What was IMI-GetReal?

Public-Private partnership
RWE use throughout drug lifecycle
3-year project

Aim:

Categorize practical issues encountered in accessing and using RWE in CER



Methods:

Qualitative analysis of case study reports
Consensus-seeking amongst co-leads

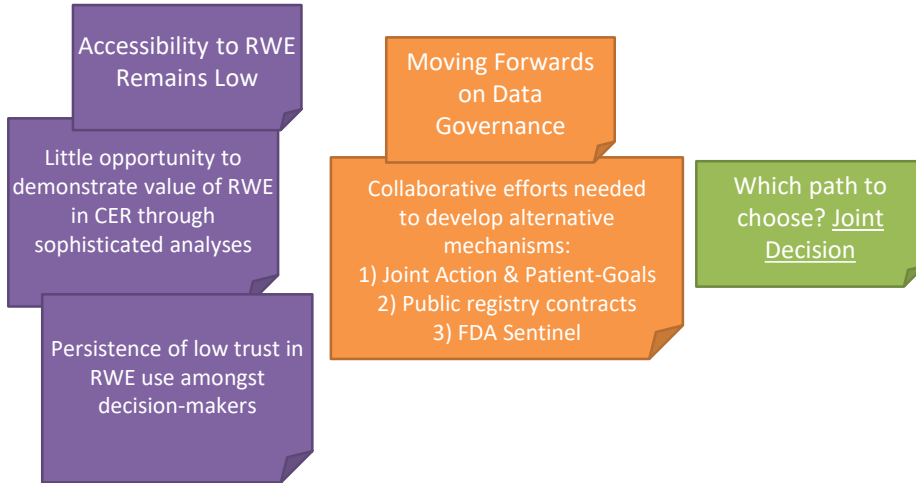


Practicalities of RWE use in CER

1. Accessing Individual Patient Data (IPD):
 - RCTs: access to 41/43 (95%) studies
 - RWE repositories: access to 7/20 (35%) repositories
2. Alternatives to IPD from RWE: Aggregate Data
 - Advantages: more accessible
 - Disadvantages: limited data on covariates
 - Remote querying of IPD to report AD
3. Methodological challenges in using IPD from RWE:
 - Making datasets research-ready
 - Differences in definitions of outcome measures (RCT vs. RWE)



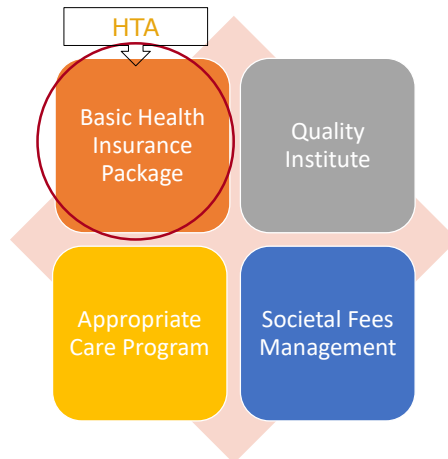
Implications for decision making?



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Where to next? ZIN's Vision



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RWE for New HTA Methodology

Indication: ... static melanoma (post-)effectiveness of immunotherapies

Proac Elec Rec

Patient assess effectiveness

• Preliminary discussions on scope etc.

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International Initiatives & Future Directions

- **EUnetHTA WP5B:** Core datasets for HTA registries
 - **EMA Adaptive Pathways:** Iterative evidence development & assessment
 - **EMA-EUnetHTA Joint Work Plan:** increased HTA/reg. collaboration on RWE
 - **IMI-GetReal I & II:** RWE use for clinical effectiveness of (Alzheimer's) drugs
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- **REPEAT Initiative:** Reliability and reproducibility of results from RWE studies
 - **NEWDIGS:** Drug development paradigms (incl. AP)
 - **ISPOR/ISPE Special Task Force:** Good procedural & reporting practices for RWE studies
 - **INAHTA RWE Task Force:** Global HTA standpoints on RWE

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Thank you for your attention.

Questions?

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