

# Assessing the value of (new) Health Technologies:

## The use of the different types of data along the lifecycle of technologies

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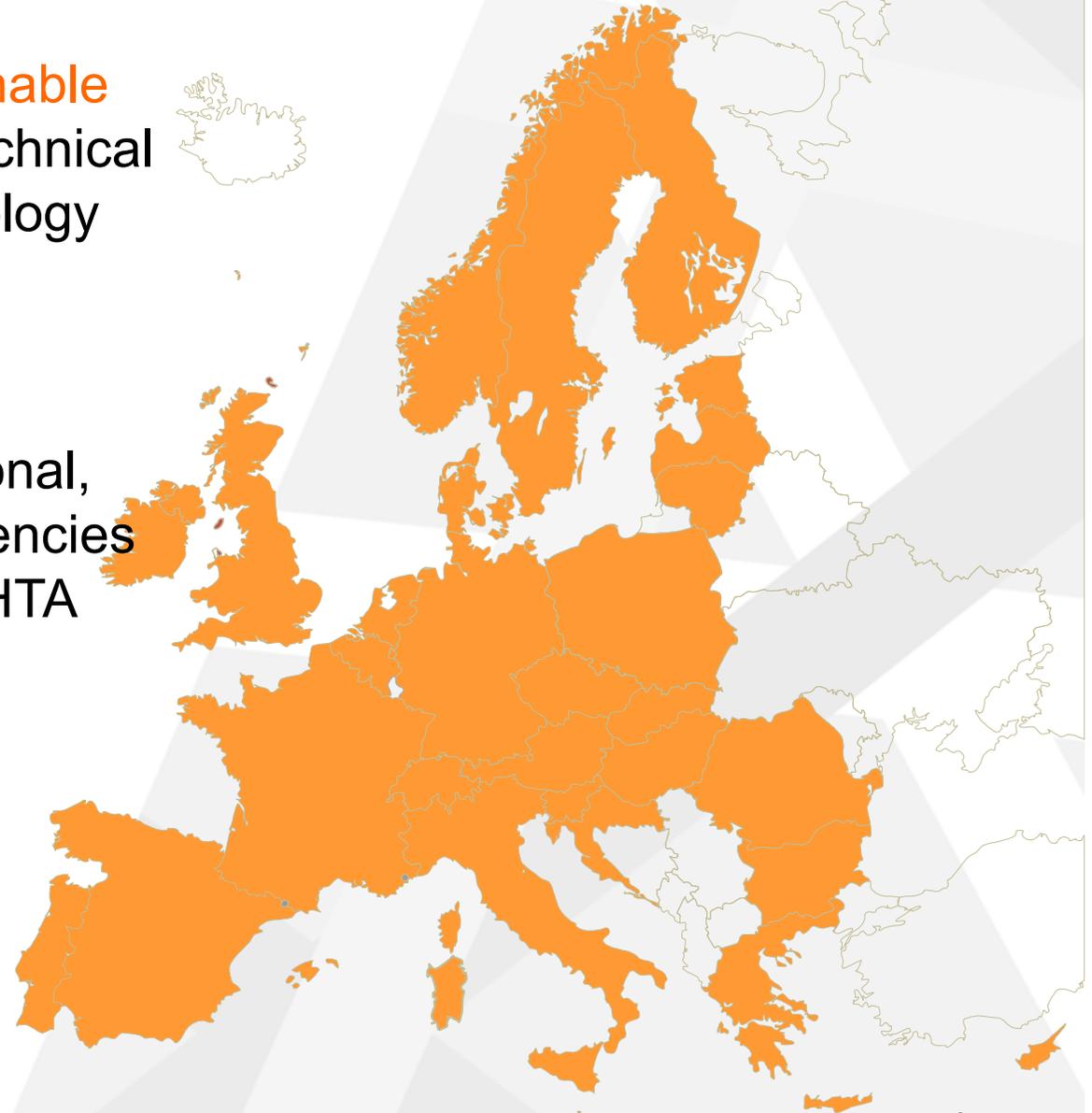
Tallinn, July 3, 2017

# EUnetHTA JA3 (2016-2020)

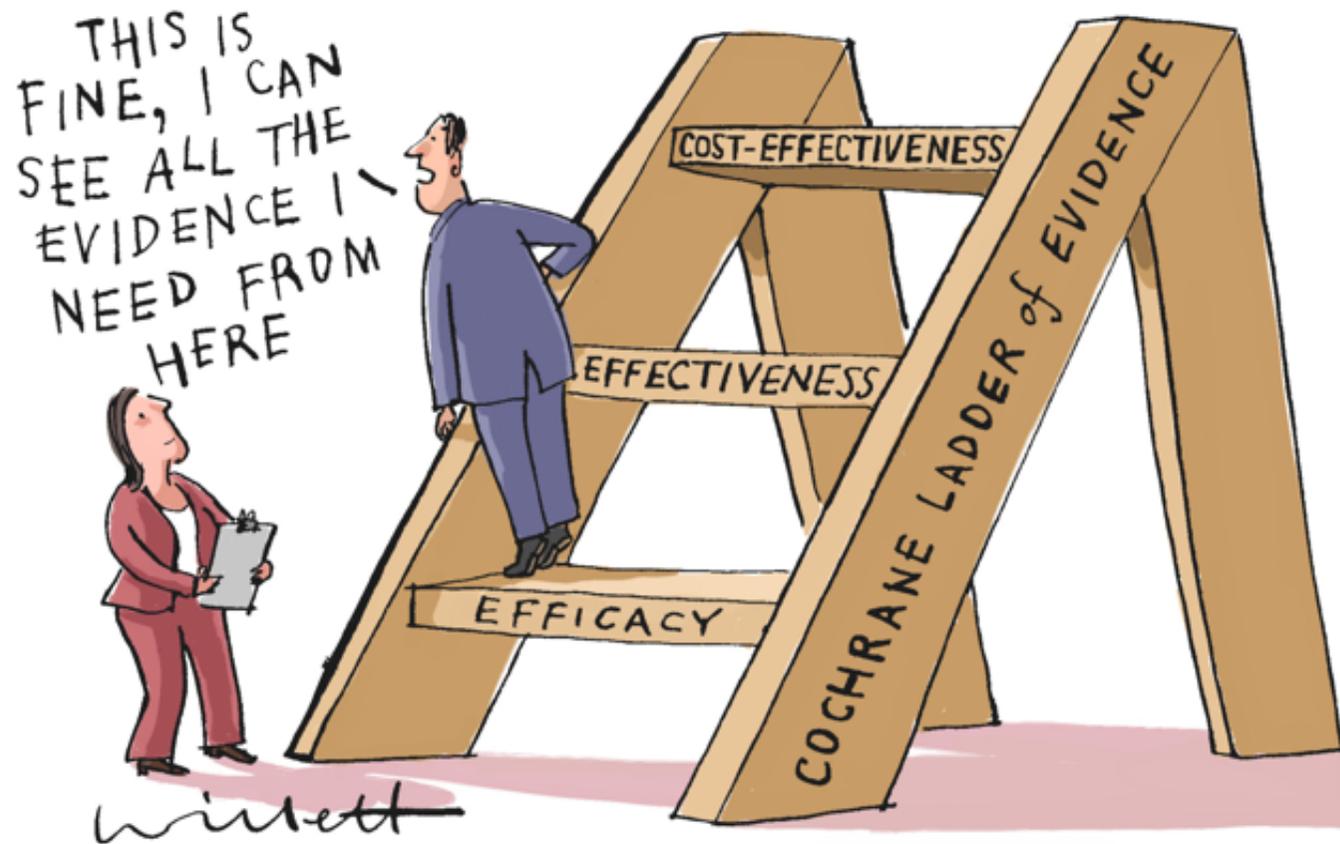
Aims to contribute to a sustainable model for the scientific and technical cooperation on Health Technology Assessment (HTA) in Europe

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)

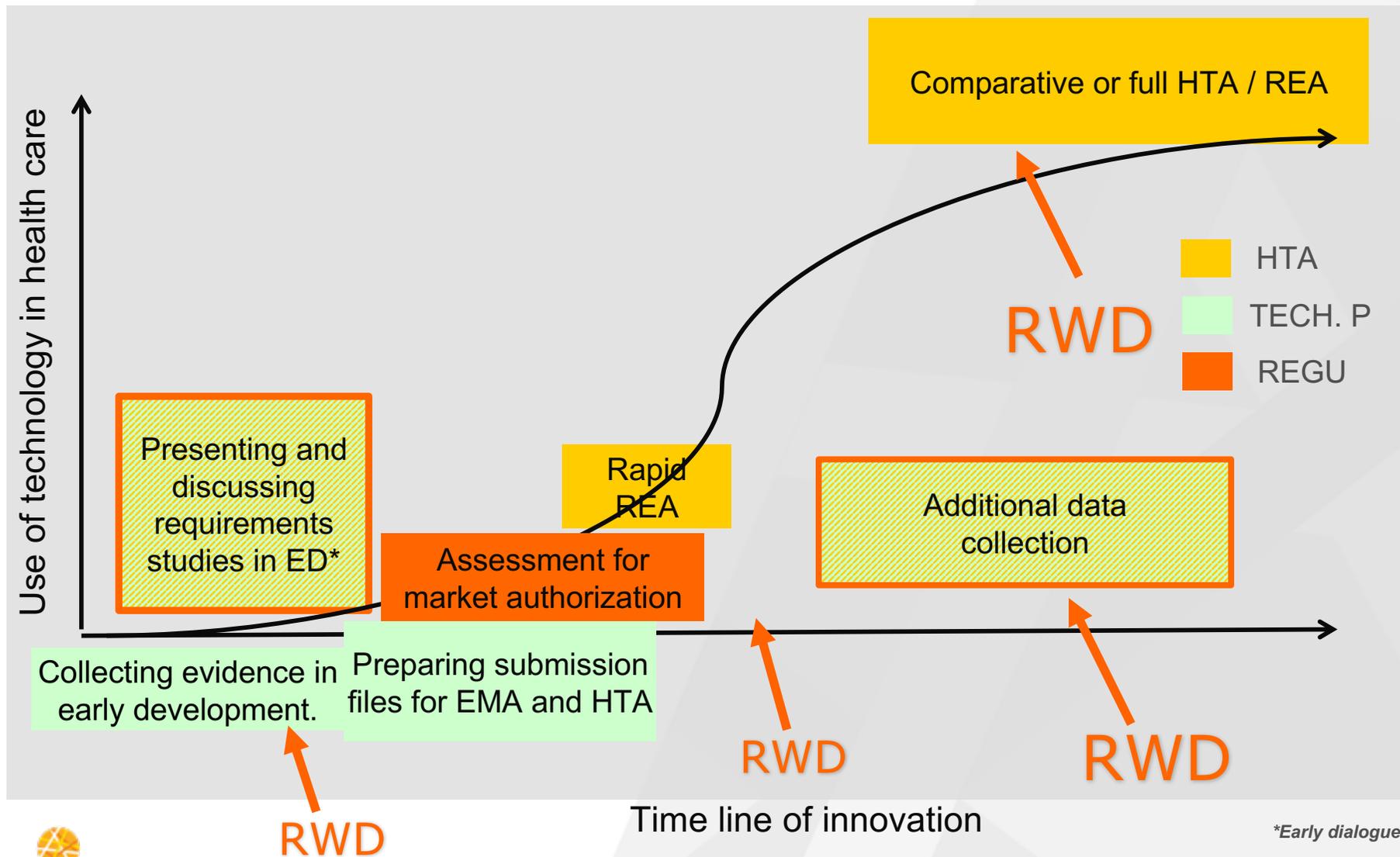


# Is there an efficacy-effectiveness gap?



The observed discrepancy between effects of a health intervention in routine clinical practice as compared with the effects demonstrated in randomised controlled clinical trials. (Adapted from Eichler et al., 2011)

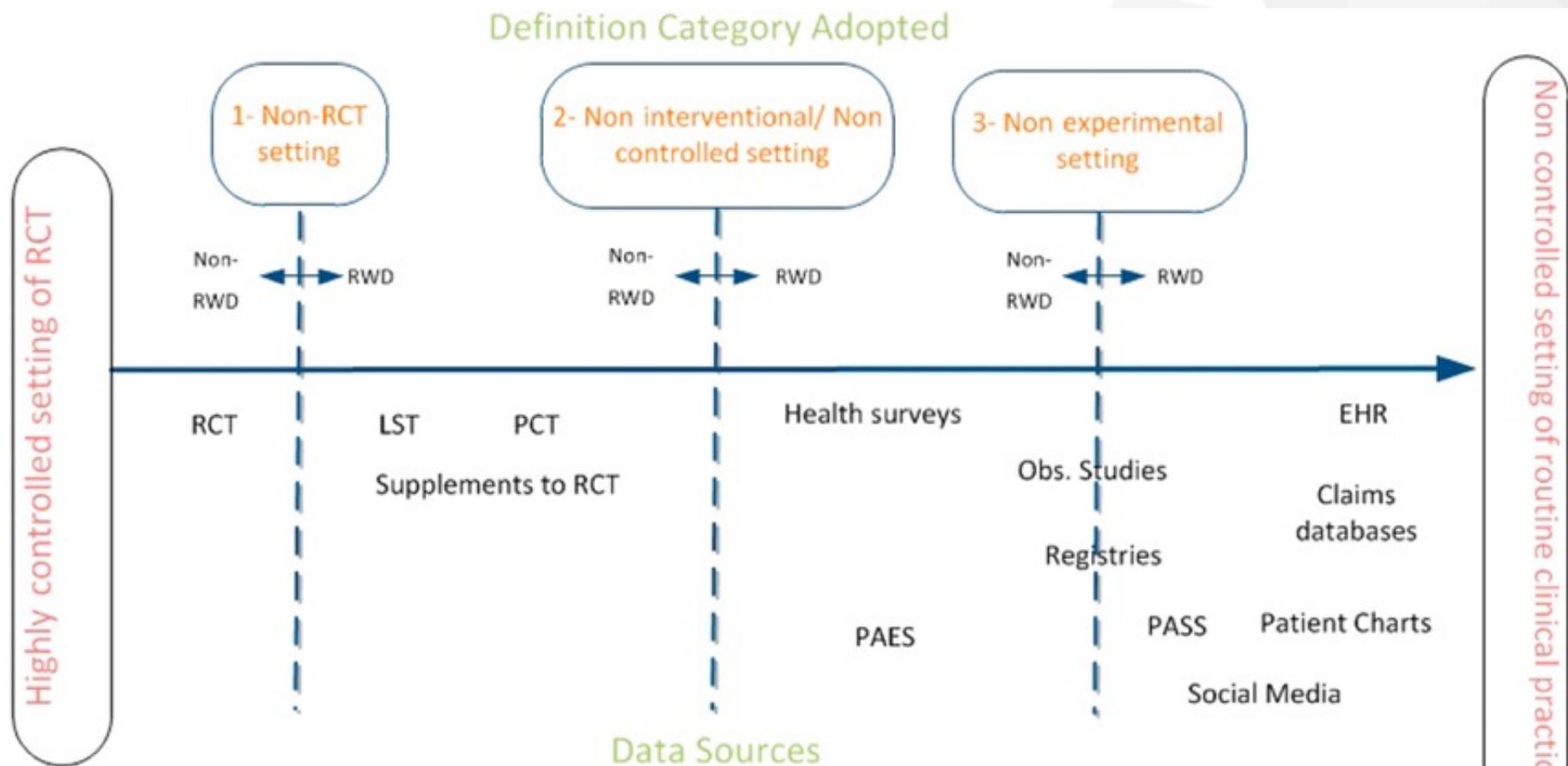
# RWD in the life cycle of technologies



# Which questions do we need to address?

- **What are RWD?**
  - Non-RCT data versus experimental data or EPR data
- **How are RWD currently being used by HTA organisations?**
  - In relation to the different types of context
  - Guidelines on RWD versus real use RWD in HTA reports
- **What are possible ways forward but also hurdles?**
  - Access to RWD for research
  - *Quality of RWD*
  - *Usability of RWD for decision-making on pricing and reimbursement*

# Definition of RWD



Legend: RWD = Real-World Data; RCT = Randomised controlled Clinical Trial; LST = Large Simple Trial; PCT = Pragmatic Clinical Trial; PAES: Post-Authorisation Efficacy Study; PASS = Post-Authorisation Safety Studies; Obs. Studies = Observational studies; EHR = Electronic Health Record.

Makady A, de Boer A, Hillege H, Klungel OH, Goettsch WG,. What Is Real-World Data (RWD)? A Review of Definitions Based on Literature and Stakeholder Interviews. Value in Health 2017; in press

# HTA policies on RWD

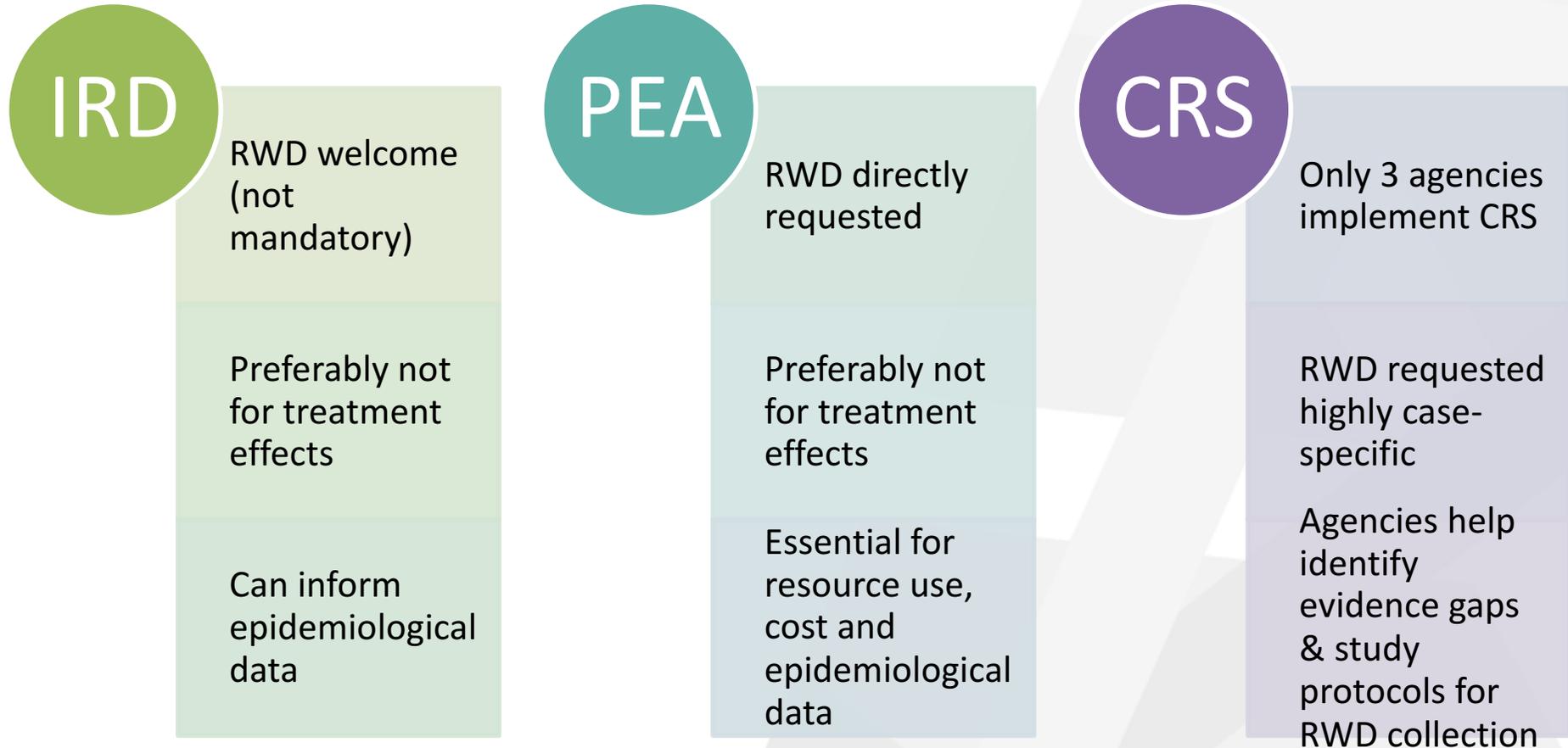
## Data Collection

- 6 Agencies:
  - TLV, NICE, IQWiG, HAS, AIFA, ZIN
- Literature Review
  - 14 policy documents
  - 6 academic documents
- Semi-structured Interviews
  - Senior HTA assessors & R&D officers

## Contexts Assessed

- Initial Reimbursement Discussions (IRD)
- Pharmacoeconomic Analysis (PEA)
- Conditional Reimbursement Schemes (CRS)

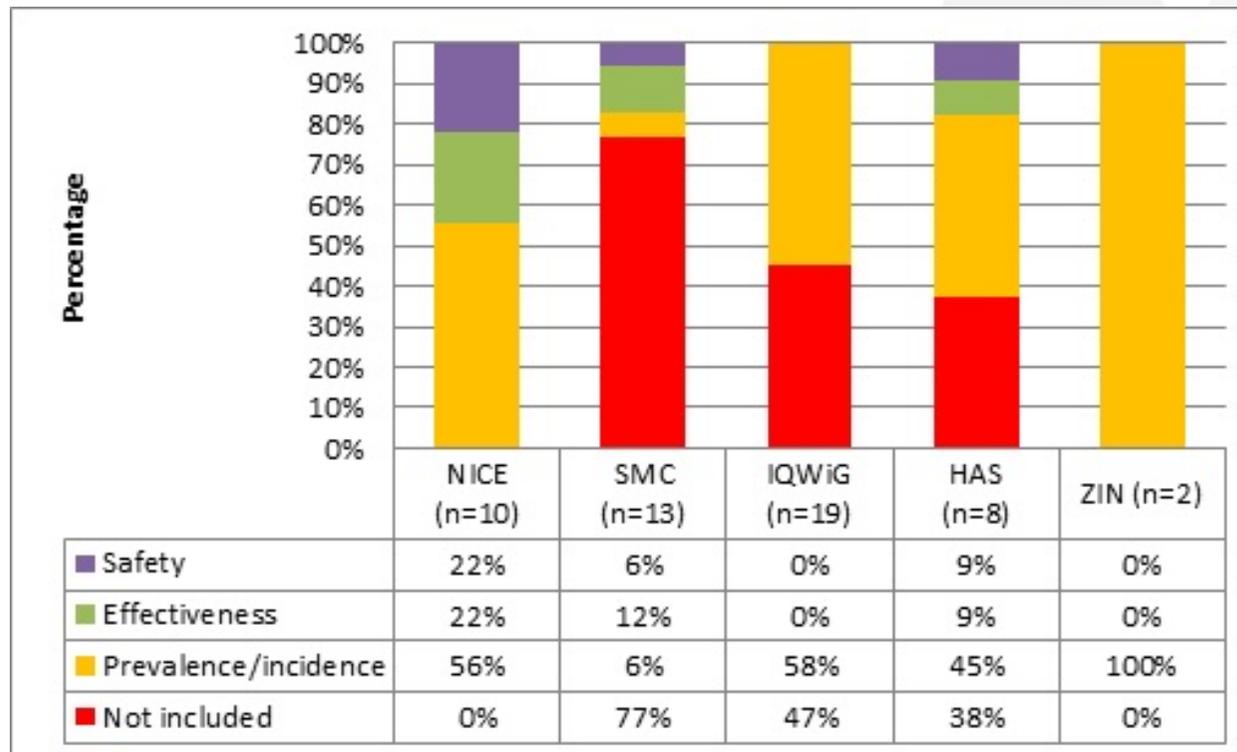
# HTA policies on RWD



*Makady A, ten Ham R, de Boer A, Hillege H, Klungel OH, Goettsch WG. Policies for Use of Real-World Data in Health Technology Assessment (HTA): A Comparative Study of Six HTA Agencies. Value in Health 2017; 20: 520-5323*

# RWD use in HTA Practice

## Relative Effectiveness Assessments (REAs)



*Makady A, van Veelen A, Jonsson P, Moseley O, d'Andon A, de Boer A, Hillege H, Klungel OH, Goettsch WG,. Use of Real-World Data in Health Technology Assessment (HTA) Practice: A Comparative Study of 5 HTA Agencies. HTAi Rome (2017).*

# Access to RWD in practice: IMI-GetReal's Experiences

## 1. Accessing Individual Patient Data (IPD):

- RCTs: access to 41/43 (95%) studies
- RWE repositories: access to 7/20 (35%) repositories
- Overall impression: varied greatly for each of 7 case studies

## 2. Alternatives to IPD from RWE: Aggregate Data

- Advantages: far more accessible from literature or registry holders
- Disadvantages: limited data on covariates = less robust adjustment for confounding in estimates
- Alternative? Remote querying of IPD to report AD with information on covariates

## 3. Methodological challenges in using IPD from RWE:

- Making datasets research-ready: cleaning, trimming, imputing values
- Differences in definitions of outcome measures (RCT vs. RWE)

*Makady A, Stegenga H, Ciaglia A, Debray TPA, Lees M, Happich M, Ryll B, Abrams K, Jonsson P, Garner S, Thwaites R, Goettsch WG. Practicalities of Using Real-World Evidence in Comparative Effectiveness Research: Learnings from IMI-GetReal. HTAi Rome (2017).*

# Implications for Decision-Making

Accessibility to RWE  
Remains Low

Little opportunity to  
demonstrate value of RWE  
in CER through  
sophisticated analyses

Persistence of low trust in  
RWE use amongst  
decision-makers

Moving Forwards on  
Data Governance

Collaborative efforts needed:  
to develop alternative  
mechanisms:  
1) Joint Action & Patient-Goals  
2) Public registry contracts  
3) FDA Sentinel

Which path to choose?  
Joint Decision

Goal: Better value to  
society in clinical  
practice!

*Makady A, Stegenga H, Ciaglia A, Debray TPA, Lees M, Happich M, Ryll B, Abrams K, Jonsson P, Garner S, Thwaites R, Goettsch WG. Practicalities of Using Real-World Evidence in Comparative Effectiveness Research: Learnings from IMI-GetReal. HTAi Rome (2017).*

# Conclusions

- **Real World Data will become important over the life-cycle**
  - Should we remain to use the term RWD?
  - More clarity and insight on the use of RWD in HTA practice is necessary
  - Access issues remain important
  - What can we do in working together?
- **Quality of RWD remains a crucial issue**
  - Transparent reporting of RWD data studies
  - Guidelines for interpretation of RWD should be implemented
  - Acceptability of RWD for decision-making needs more interaction with the final decision-makers

**Thank you**  
**Any questions?**

