



# HTA acceptance of RWE in healthcare decision making: a critical review from seven countries

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Case-study

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## Authors

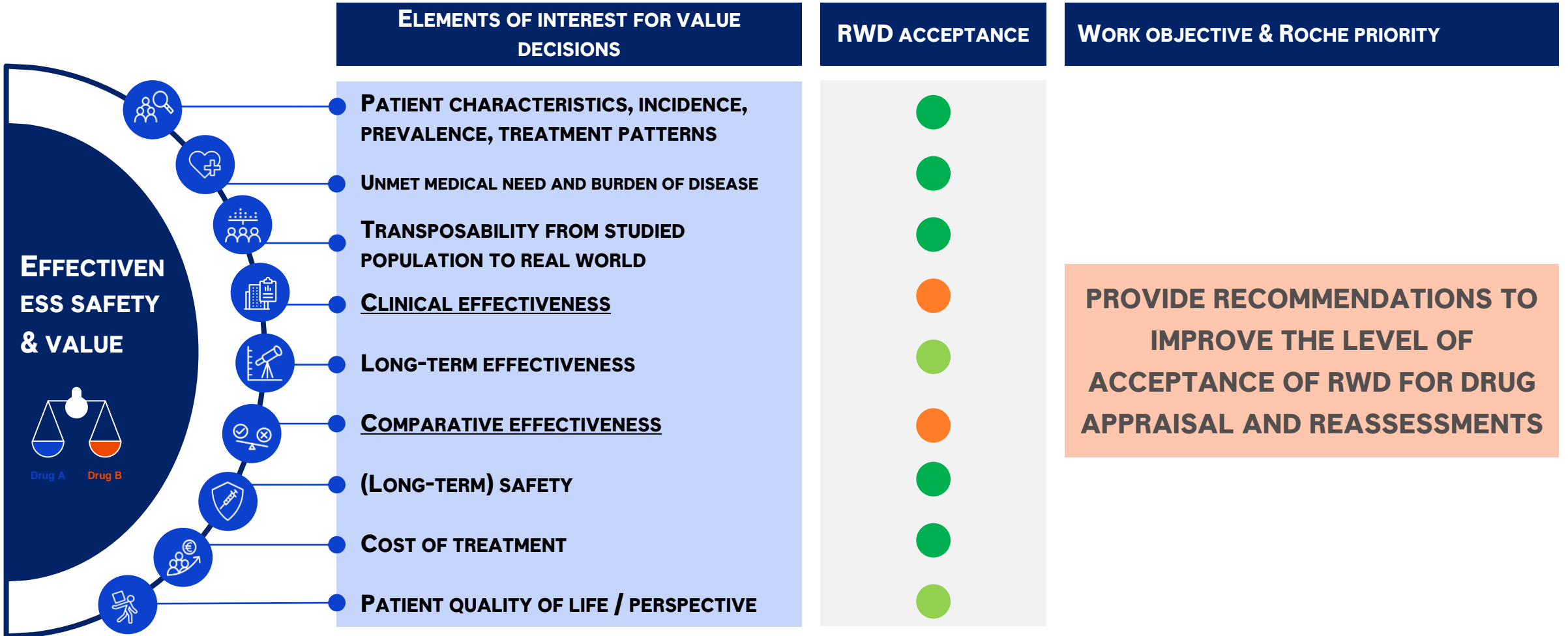
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# Clinical and comparative effectiveness RWD have limited acceptance during HTA discussions



Sources: 1. Makady, A., et al. (2018) Pharmacoeconomics 36:359-68; 2. Kent, S., et al. (2021) Pharmacoeconomics 39:275-85; 3. Bullement, A., et al. (2020) Int J Tech Assess Health Care 36:388-94; 4. PDP 2018 Value Story.

Broad acceptance: ●, Acceptance: ●, Limited acceptance: ●  
 EMR: Electronic medical record, HTA: Health technology assessment, QoL: Quality of life, RWD: Real World Data

# Our objective was to describe HTA acceptance of RWD across 7 countries



Methodology<sup>1</sup>



## METHODOLOGY



**BASED ON HTA BODIES'**

**METHODOLOGICAL GUIDELINES**

Knowledge of each HTA bodies **theoretical requirements for RWD** in order to inform the analysis of their real uses and acceptance

**BASED ON ROCHE AFFILIATES' EXPERIENCE**

Capitalization on the knowledge of each HTA body: **submission process in real practice, assessment and appraisal of RWD** in order to identify synergies and/or differences and "best practices"

**BASED ON ROCHE AFFILIATES' CASE-STUDIES**

Illustration of the findings with different case-studies in order to **identify the acceptance level of RWD in terms of assessment & appraisal**

**BASED ON A LITERATURE REVIEW**

**Support and supplement the findings** with those found in the literature review

# Five key findings

# Local methodological guidance and templates are not used by the majority of HTA bodies



Key findings (1/5 & 2/5)

## MAIN RESULTS<sup>1</sup>



## KEY LEARNINGS



### 1. ABSENCE OF LOCAL METHODOLOGICAL GUIDANCE / TEMPLATES

HTA BODIES THAT DO NOT HAVE A METHODOLOGICAL GUIDANCE

4/7



HTA BODIES WITH METHODOLOGICAL GUIDANCE



“these documents do not constitute formal NICE guidance or policy” (NICE,2022)  
“should be seen as a methodological guideline. [...] This is not a binding document.”  
(HAS 2021)

HTA BODIES THAT DO NOT HAVE TEMPLATES

6/7



HTA BODIES WITH TEMPLATES



- *When available, methodological guidance is considered useful*
- *Guidelines contain information mostly about outcomes, study design, data sources, protocol requirements, definition of the study population, external arms, biases minimization,...*

- *Templates are helpful in order to describe and structure the methodological requirements in the protocol and the report (including checklists such as STROBE).*

### 2. ABSENCE OF GUIDANCE FOR RWD ACCEPTANCE

HTA BODIES THAT DO NOT HAVE A GUIDANCE FOR RWD ACCEPTANCE

7/7

- *None of the HTA bodies have any guidance aimed at specifying when RWD is expected and accepted, and for which study objectives.*

# A variety of RWD study designs can be submitted to HTA bodies to address the expected outcomes



Key findings (3/5)

## MAIN RESULTS<sup>1</sup>



## KEY LEARNINGS



### 3. HIGH VARIETY OF RWD STUDY DESIGNS, DEPENDING ON THE SCOPE AND OBJECTIVE OF THE ASSESSMENT



#### TYPE OF ASSESSMENTS

High variety

TOP

- EFFICACY GAP BETWEEN RCT & RWD
- COMPARATIVE EFFECTIVENESS
- RELEVANCE OF PRODUCT EFFECT



#### TYPE OF RWD SOURCES

High variety

TOP

- HEALTHCARE DATABASES INCL. EHR
- PATIENTS' REGISTRIES



#### HTA BODIES THAT PREFER LOCAL RWD SOURCES Majority



- *RWD on efficacy gap between RCT and RWD is the most used.*



- *Multiple sources are considered to provide RWD:
  - Patient registries & healthcare databases including EHRs are the most widely used.*



- *Most HTA bodies prefer local RWD sources to put results into perspective in local current practice and ensure their transposability to the eligible population.*

### 3. ABSENCE OF RWD SOURCES' INDEX

HTA BODIES THAT DO NOT HAVE RWD SOURCES' INDEX

4/7



- *RWD sources' index exist but are not exhaustive and detailed for each therapeutic area in France and Brazil.*

# Lack of external review of protocols and results may limit Payer/HTA acceptance - few Agencies engage external societies during review and assessments



Key findings (4/5)

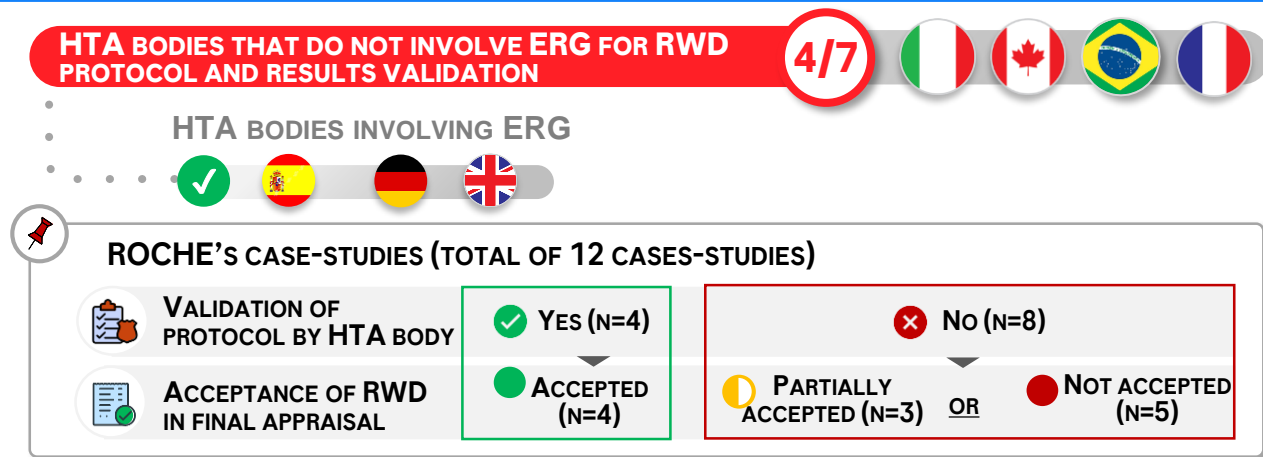
## MAIN RESULTS<sup>1</sup>



## KEY LEARNINGS



### 4. ONLY HTA BODIES WITHIN THE UK, GERMANY AND SPAIN INVOLVE AN EXTERNAL REVIEW GROUP (ERG) FOR STUDY ASSESSMENT



- Most of the HTA bodies do not involve an ERG involved during the assessment of the protocol & study report, except in the United-Kingdom, Germany and Spain. Only the United-Kingdom considers their conclusions in its final appraisal.
- Roche's experience shows, through 12 case-studies, that validation of RWD study protocol by Health Authority leads to improved RWD acceptance. Involvement of an ERG in protocol review and validation by HTA bodies could be considered for all HTA bodies.

### 4. MOST COUNTRIES HAVE EXPERTS OR ACADEMIC RESEARCH GROUPS INVOLVED IN DIFFERENT ASPECTS OF RWE APPRAISAL



- KOLs or experts are involved in RWD recognition in all countries, but their roles differ:
  - Publishing on the use of RWD in most countries
  - Submission and appraisal steps in half of the reviewed countries
  - Advocating on the use of RWD in a few countries

# Consistent early HTA engagement would standardize benefit requirements and acceptance of RWD

Key findings (5/5)



## MAIN RESULTS<sup>1</sup>



## KEY LEARNINGS



### 5. EARLY CONSULTATIONS WITH THE HTA BODY ARE POSSIBLE IN DIFFERENT CASES

#### COUNTRIES THAT CAN HAVE EARLY CONSULTATION WITH HTA BODIES

5/7



**SPAIN**  
No possibility to apply for early meetings.



**ITALY**  
Possibility to apply for early meetings but difficult to obtain and plan in practice.

- *In most countries (i.e., United-Kingdom, Canada, Brazil, Germany and France), early consultations with the HTA body are authorized, but they don't relate to RWD specifically.*
- *In countries where early consultations are not possible or hardly accessible, RWD acceptance seems to be lower:*
  - *Early meetings might have helped to discuss with the HTA body about the RWD study submission's objective, approach and utility of the evidence being generated.*



# Discussion

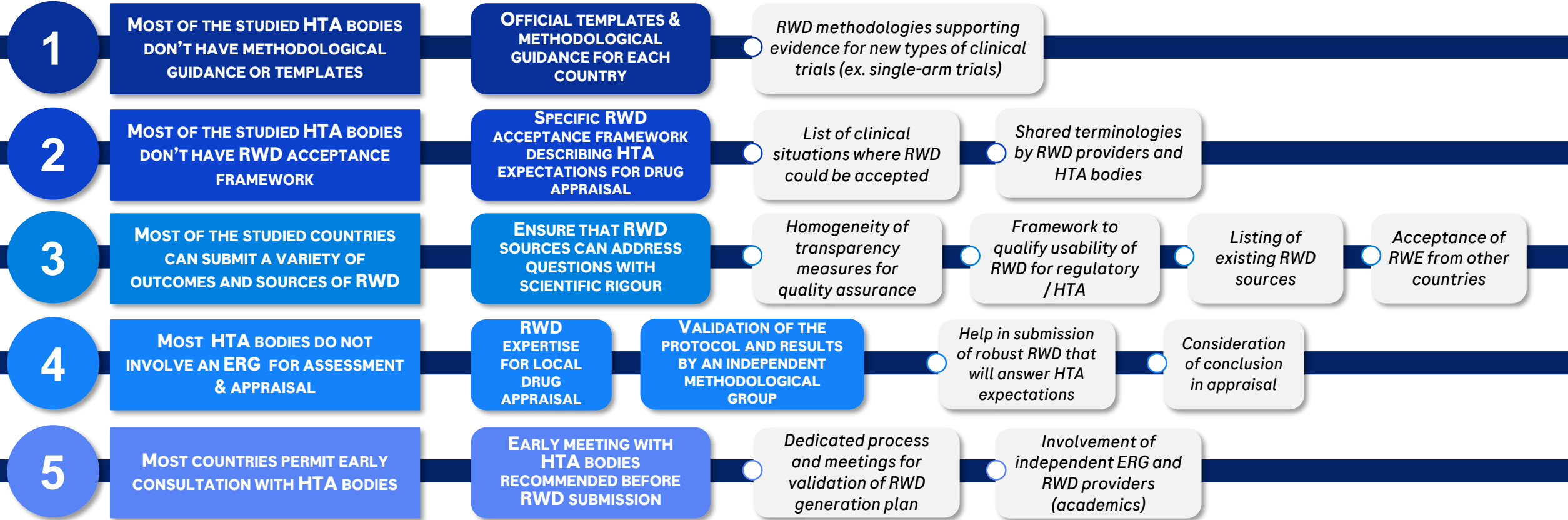
# Our work resulted in the following recommendations



## Discussion

### KEY FINDINGS

### RECOMMENDATIONS



“ Would you agree with these recommendations for your country?  
How would you define RWD acceptance? ”

**Doing now what patients need next**

***Thank you for your attention***