



Four Pillars to shape comprehensive RWE guidance for HTA/Payers – building on existing work

Karen Facey, PhD CStat

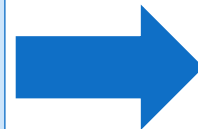
Consultant to RWE4Decisions Secretariat



Novartis reflections on RWE4Decisions Discussions in 2020

RATIONALE

- Literature indicates that assessment methods and policies on acceptance of RWE vary across decision-makers due to a range of issues
- Many national HTA bodies don't have clear guidance about generating RWE or how it will be critically assessed, and nothing endorsed across HTA bodies
- **Need for clear guidance for HTA bodies, industry, and other stakeholders**



PROJECT AIMS

- Objective 1: Summarize barriers to RWD/RWE uptake by HTA agencies and Payers (*emphasis on papers with feedback from HTAs/Payers in the EU*)
- Objective 2: Identify relevant solutions available from trusted institutions across HTA agencies, regulators and the scientific community (*including multistakeholder initiatives*)



Methodology to explore perspectives and guidance on use of RWE in HTA and regulatory decision-making

Novartis Team – scoping/pragmatic literature search

- In April 2021, review of publications about issues relating to:
 - Papers including range of HTA/Payers' perspectives about use of RWE
 - Guidance on use of RWE in decision-making
- Quick search of PubMed for recent papers and snowballing review of associated references
- Targeted website searches to identify guidance and reports
 - HTA bodies (NICE, HAS, IQWiG, TLV, CADTH) + PCORI
 - Multi-stakeholder groups involved in RWE initiatives (GET REAL RWE-navigator, ISPOR, ISPE)
 - Regulatory agencies (FDA, EMA)
- Key information was extracted for each document and is available in a full presentation deck and shared with panelists

KF – critical review and clarifications

Objective 1

Summarize barriers to RWD/RWE uptake by HTA agencies and Payers

What do HTA/Payers in the EU think about use of RWE?





1. RWD already accepted for some HTA questions

Key papers with the EU HTA/Payer engagement

Oortwijn et al. (2018; 2019) HTAi Global Policy Forum

1. How to deal with the inevitable: Generating RWD and using RWE for HTA purposes – from theory to action
2. RWE in the context of HTA processes – from theory to action (background paper)

Facey et al. (2020) RWE4Decisions

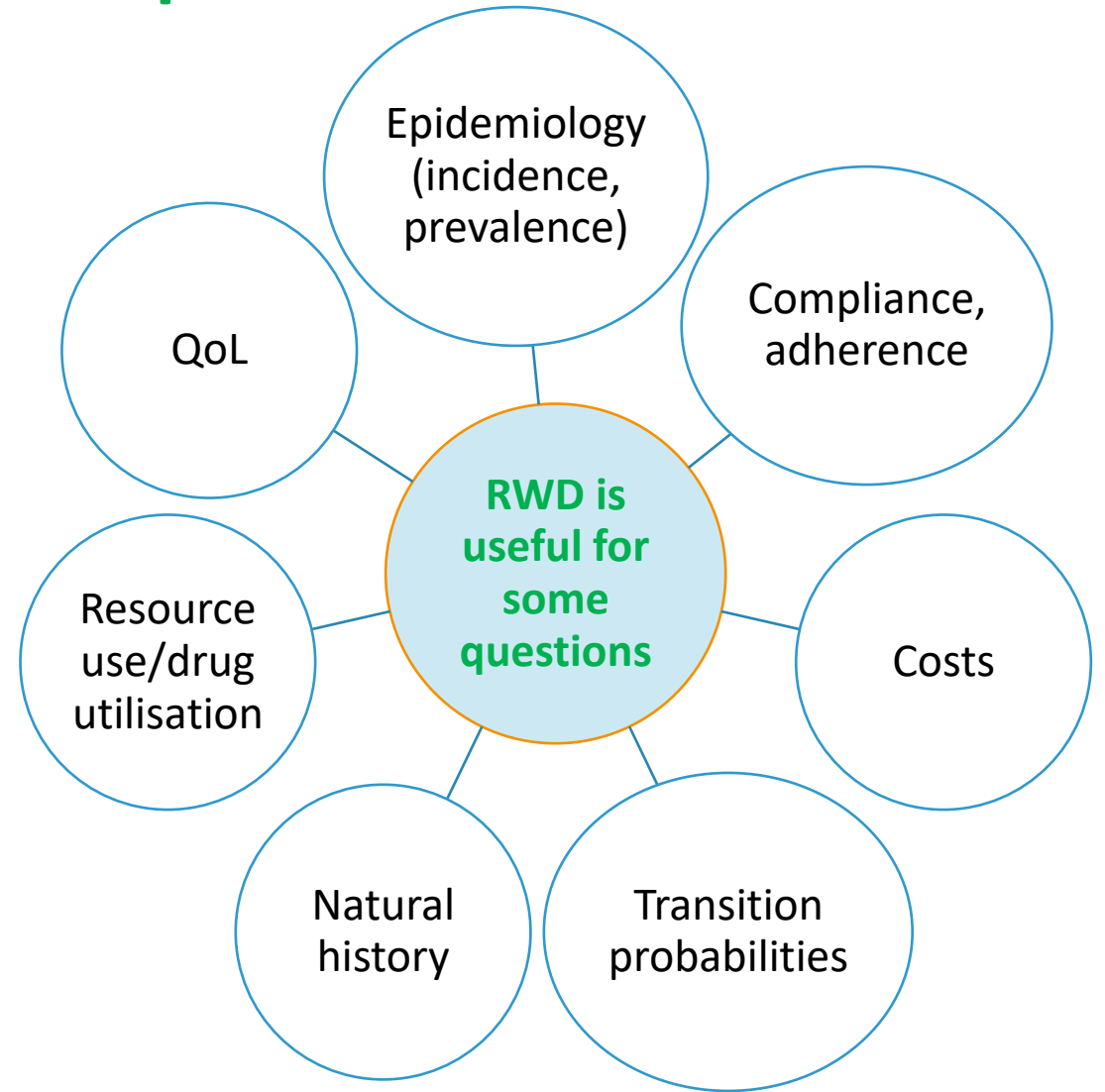
3. RWE to support Payer/HTA decisions about highly innovative technologies in the EU – actions for stakeholders

Makady et al. (2017; 2018)

4. Using RWD in HTA practice: A comparative study of 5 HTA agencies
5. Policies for use of RWD in HTA: A comparative study of 6 HTA agencies

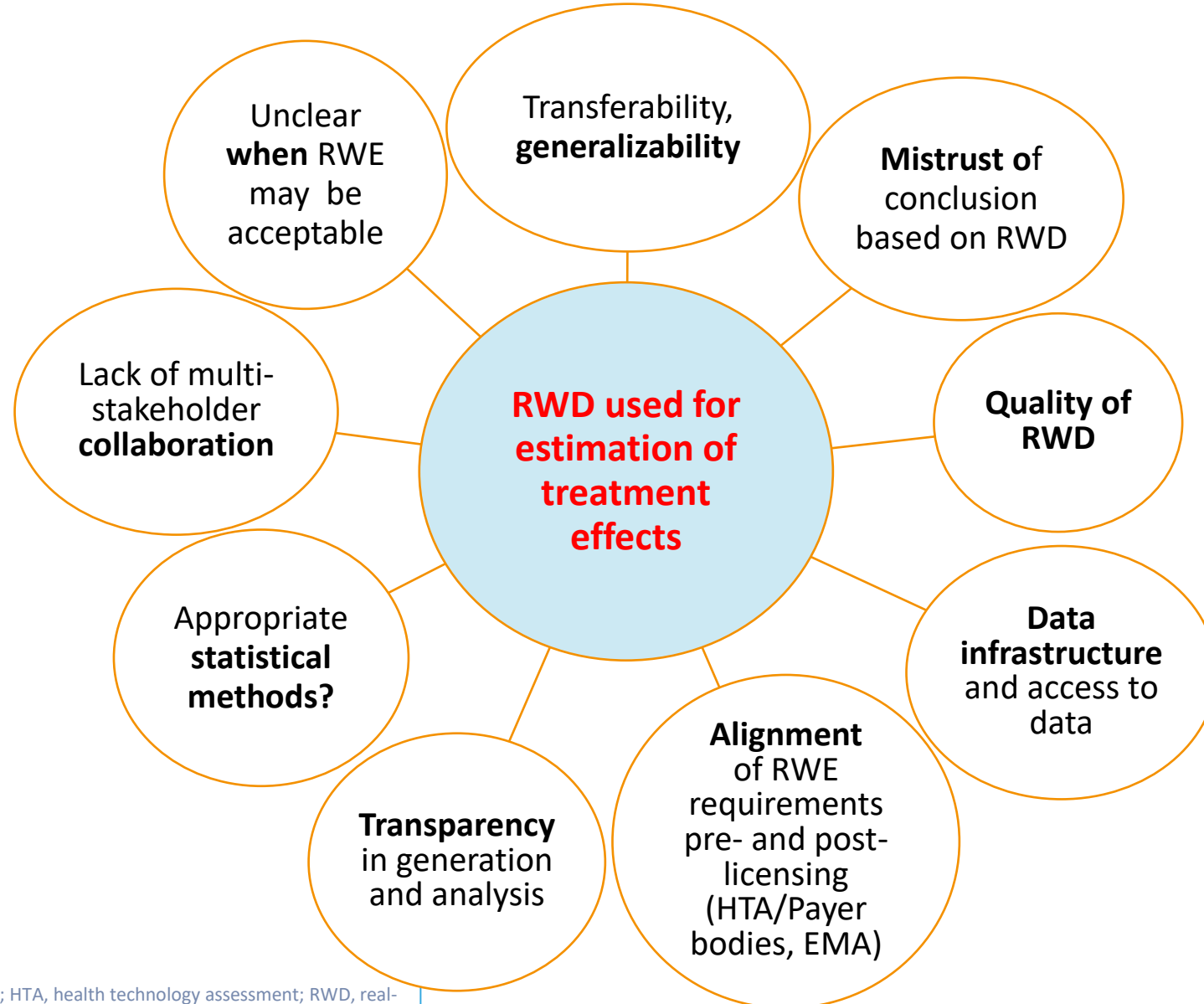
Sievers et al. (2021)

6. RWE: Perspectives on challenges, value and alignment of regulatory and national HTA data collection requirements





2. Issues related to use of RWE to demonstrate treatment effects





Key papers including HTA/Payer feedback with issues and themes (1/3)

Reference	Issues reported	Themes
Oortwijn et al. 2019 Link	<ul style="list-style-type: none">▪ RWE/RWD quality and acceptability▪ Data quality, acceptability, interoperability and replicability across different data sets▪ Disparate data governance, standards, privacy standards hampering access▪ Bias▪ HTA does not have skills to advise on RWE studies or critically appraise them▪ Trust and transparency▪ Relevance, what type of HTA questions RWE is appropriate to answer▪ Increased collaboration with those that are capturing/analysing RWD▪ Limited standards for collaboration between stakeholders wrt RWD	Data Data Data Methodology Methodology Trust Policy & Partnerships Policy & Partnerships Policy & Partnerships
Oortwijn et al. 2018 Link	<ul style="list-style-type: none">▪ Quality of data from real-world sources▪ Data infrastructure and access to data, interoperability between different data sets▪ Transferability issues (generalizability of data from different contexts, countries etc.)▪ For which HTA questions might RWE be acceptable as fit for purpose?▪ When to use RWE across the lifecycle – no consensus	Data Data Data Policy & Partnerships Policy & Partnerships



RWE uptake by EU HTA/Payers – 4 Key Themes

Data: Availability, Governance and Quality

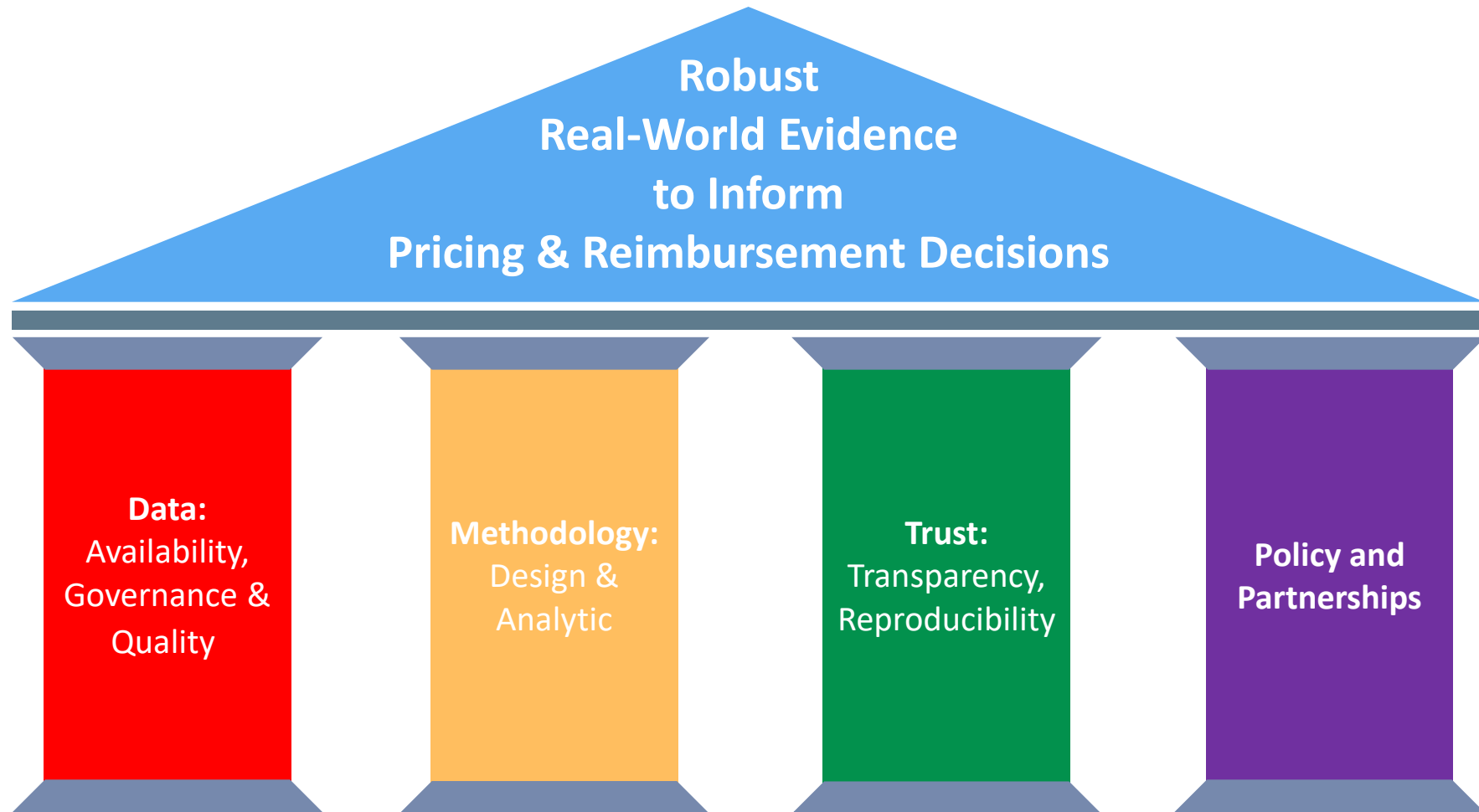
Methodology: Design and Analytic

Trust: Transparency and Reproducibility

Policy and Partnerships



RWE uptake by HTA/Payers in EU and North America – confirmed 4 Key Pillars to shape robust RWE for decision-makers





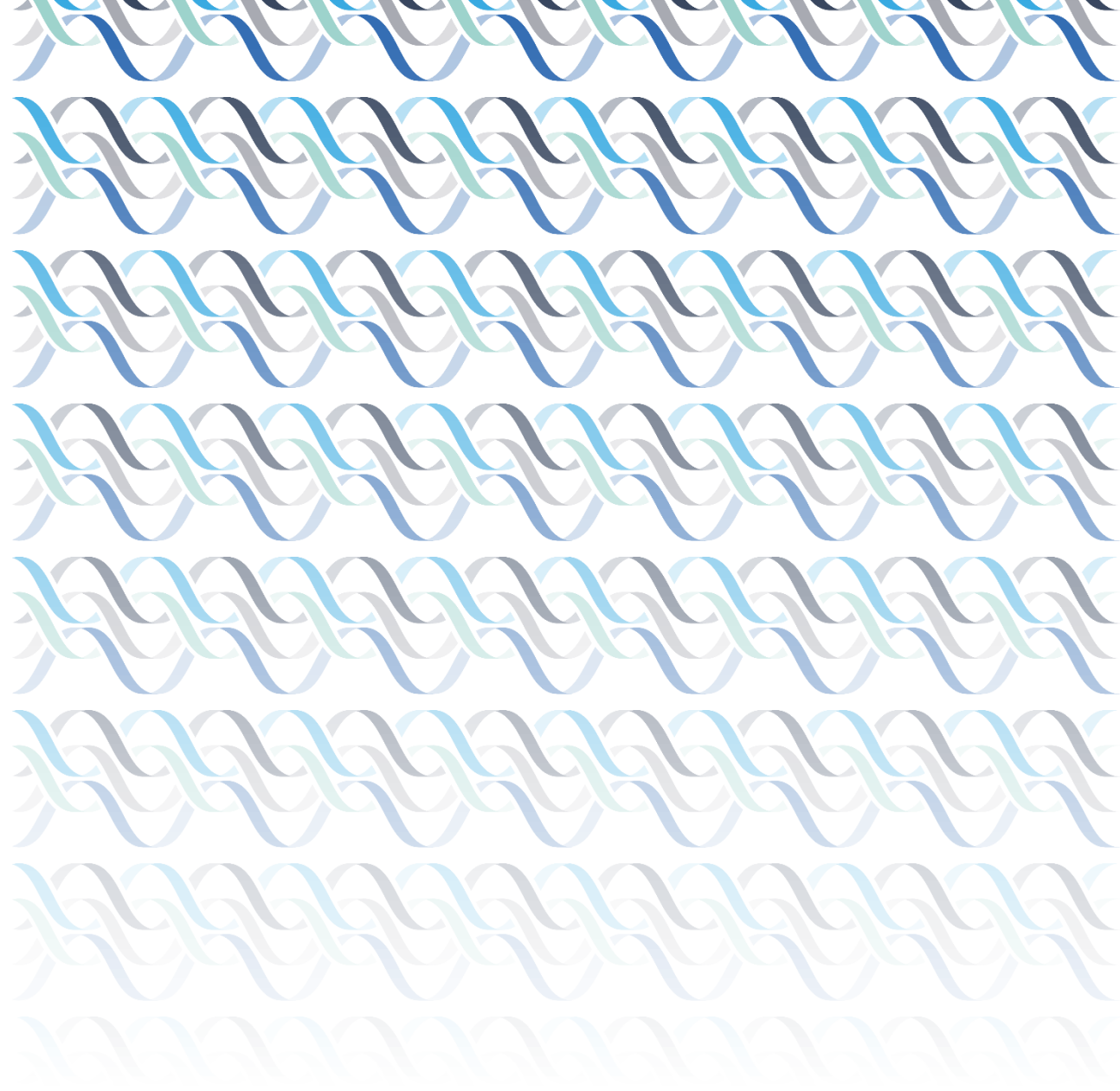
Data: Availability, Governance and Quality

Challenges

1. Poor quality of RWD including incomplete, missing data
2. Data standardization (e.g. common data model – pros and cons)
3. Timeliness of data
4. Lack of robust data and inadequate data infrastructure
5. Disparate data infrastructures, access processes and governance

Objective 2

Identify potential solutions to address HTA/Payer challenges with use of RWE





Solutions?

Data
Availability, Governance and Quality



EuNetHTA Request tool, Duke Margolis, OHDSI OMOP, EHDEN, i~HD
HMA-EMA Joint Big Data Steering Group - DARWIN EU , FDA Sentinel
Initiative & Framework, IMI-GetReal, ISPE, ISPOR, IQWiQ, ISPE, REPEAT
Initiative, Friends of Cancer Research RWE Pilot Projects, CANReValue
Data Working Group

Methodology
Design and Analytic



STRATOS, GRACE, IMI-GetReal, AHRQ, Duke Margolis, FDA, Health
Canada, ENCePP, European Commission, EUNetHTA, NICE, WHO
Guidance Documents, NICE, IMPACT HTA, ISPOR

Trust
Transparency and Reproducibility



Council for International Organizations of Medical Sciences (CIOMS)
RWE Alliance, FDA, Transcelerate Biopharma Inc., ENCePP, SPACE,
RECORD, STROBE, REPEAT, OPERAND, RCT DUPLICATE Initiative, Duke
Margolis, RWE4DECISIONS, RWE Transparency Initiative

Policy and Partnerships



Council for International Organizations of Medical Sciences (CIOMS)
FDA-Regan-Udall-Friends of Cancer-Aetion, FDA-EMA, GetReal
Institute, The Observational Medical Outcome Partnership
ISPOR/ISPE, RWE4Decision, NICE_Flatiron Health Collaboration



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Perspective

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Organized structure of real-world evidence best practices: moving from fragmented recommendations to comprehensive guidance

Ashley Jakska¹, James Wu², Páll Jónsson³, Hans-Georg Eichler⁴, Sarah Vittoe¹ & Nicolle M Gatto¹

¹Scientific Research, Aetion Inc., 5 Penn Plaza, 7th Fl., New York, NY 10001, USA

²Amgen Inc, 1 Amgen Center Drive, Thousand Oaks, CA 91320-1779, USA

³National Institute for Health & Care Excellence, Level 1A, City Tower, Piccadilly Plaza Manchester, M1 4BT, UK

⁴European Medicines Agency, Domenico Scarlattilaan 6, Amsterdam 1083 HS, Netherlands

*Author for correspondence: Tel: +1-844-823-8466; ashleyjakska@aetion.com

Decision-makers have become increasingly interested in incorporating real-world evidence (RWE) into their decision-making process. Due to concerns regarding the reliability and quality of RWE, stakeholders have issued numerous recommendation documents to assist in setting RWE standards. The fragmented nature of these documents poses a challenge to researchers and decision-makers looking for guidance on what is 'high-quality' RWE and how it can be used in decision-making. We offer researchers and decision-makers a structure to organize the landscape of RWE recommendations and identify consensus and gaps in the current recommendations. To provide researchers with a much needed pathway for generating RWE, we discuss how decision-makers can move from fragmented recommendations to comprehensive guidance.

First draft submitted: 21 October 2020; Accepted for publication: 18 March 2021; Published online: 30 April 2021



Journal of **Comparative Effectiveness Research**

ASSESS

HEALTH TECHNOLOGIES

METHODOLOGICAL GUIDE

Real-world studies for the assessment of medicinal products and medical devices

10 juin 2021



New methods for the effective use of real-world data and/or synthetic data in regulatory decision-making and/or in health technology assessment

TOPIC ID: HORIZON-HLTH-2022-TOOL-11-02

Grant

General information

Topic description

Destination

Conditions and documents

Partner search

Submission service

Topic related FAQ

Get support

Call updates


General information

Programme

Horizon Europe Framework Programme (HORIZON)

Call

[Tools and technologies for a healthy society \(Single Stage - 2022\) \(HORIZON-HLTH-2022-TOOL-11\)](#)

 [See budget overview](#)

Type of action

HORIZON-RIA HORIZON Research and Innovation Actions

Type of MGA

HORIZON Action Grant Budget-Based [HORIZON-AG]

[Open for submission](#)

Deadline model

single-stage

Opening date

06 October 2021

Deadline date

21 April 2022 17:00:00 Brussels time

RWE4Decisions

REAL WORLD EVIDENCE



Google Maps interface showing directions from New York Penn Sta. (33RD & 7th Ent) to Bloomingdales (1000 3rd Ave, 59th & 60th, New York, NY 10022). The route is 2.2 mi, about 9 mins. The directions list five steps: 1. Head southwest on 7th Ave toward W 32nd St (249 ft); 2. Turn left at W 32nd St (0.2 mi); 3. Turn left at 6th Ave/Ave of the Americas (0.5 mi); 4. Turn right at W 42nd St (0.6 mi); 5. Turn left at 3rd Ave (0.9 mi). A Street View inset shows a yellow taxi at W 32nd St.