Advancing the Use of Real-World Data & Real-World Evidence

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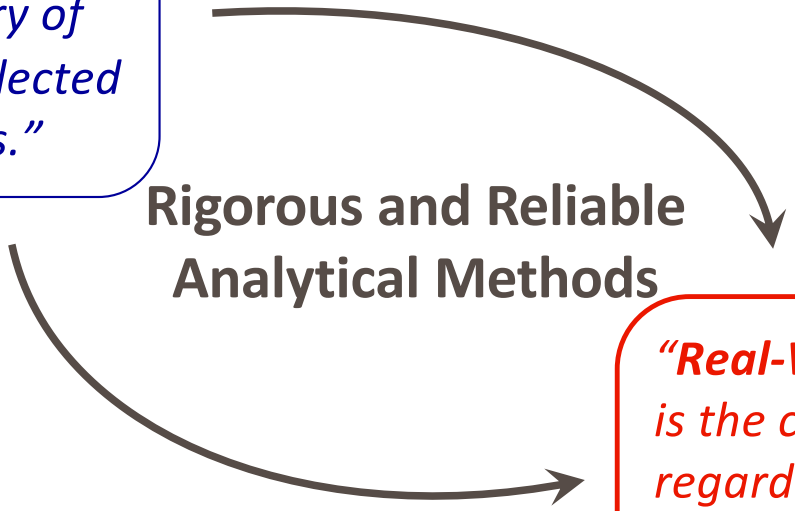
Global Regulatory Policy

Defining Real-World Data and Evidence

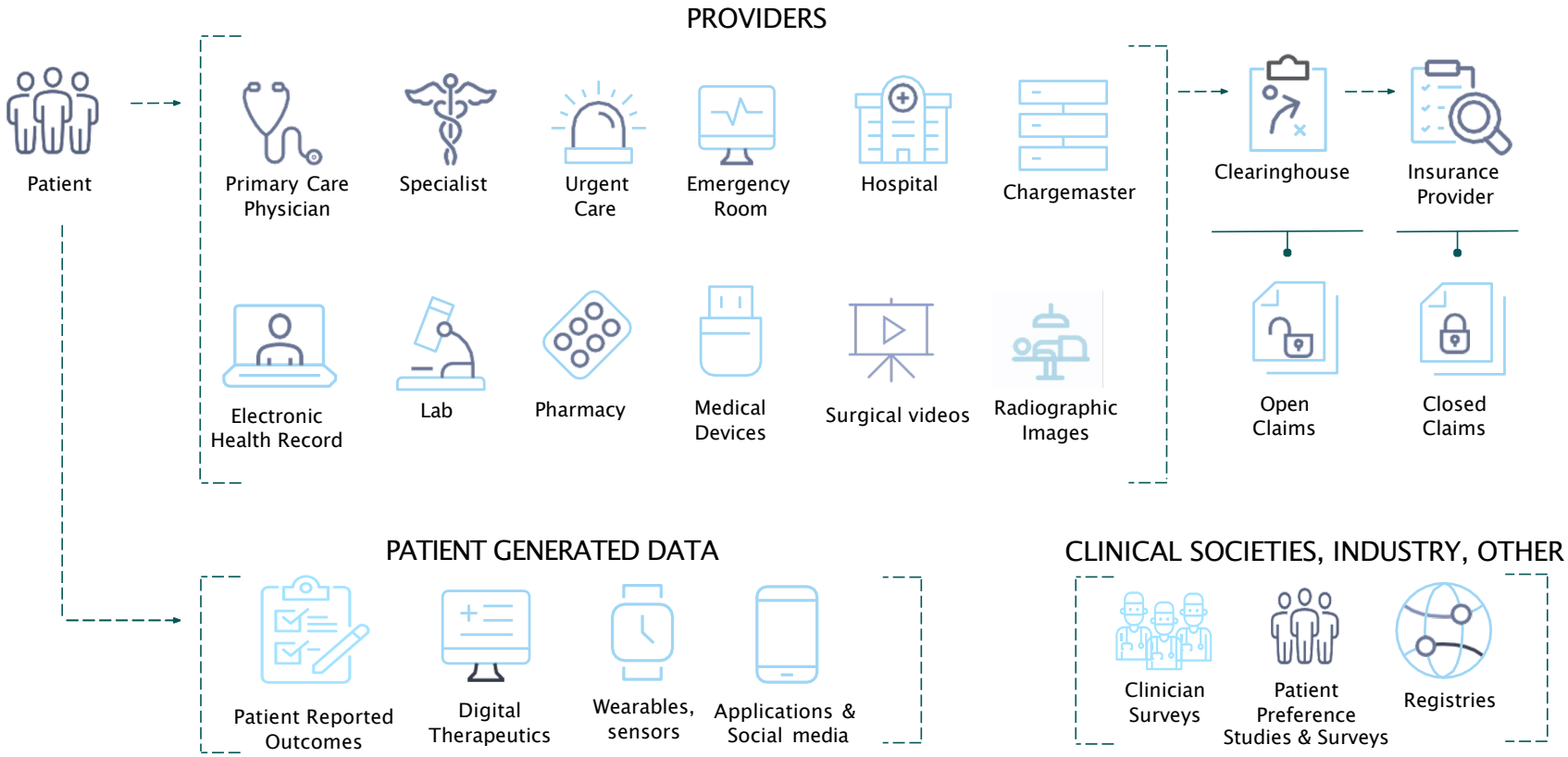
US FDA CDRH's 2017 Guidance was the first policy explicitly addressing RWD and RWE

*"**Real-World Data (RWD)** are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources."*

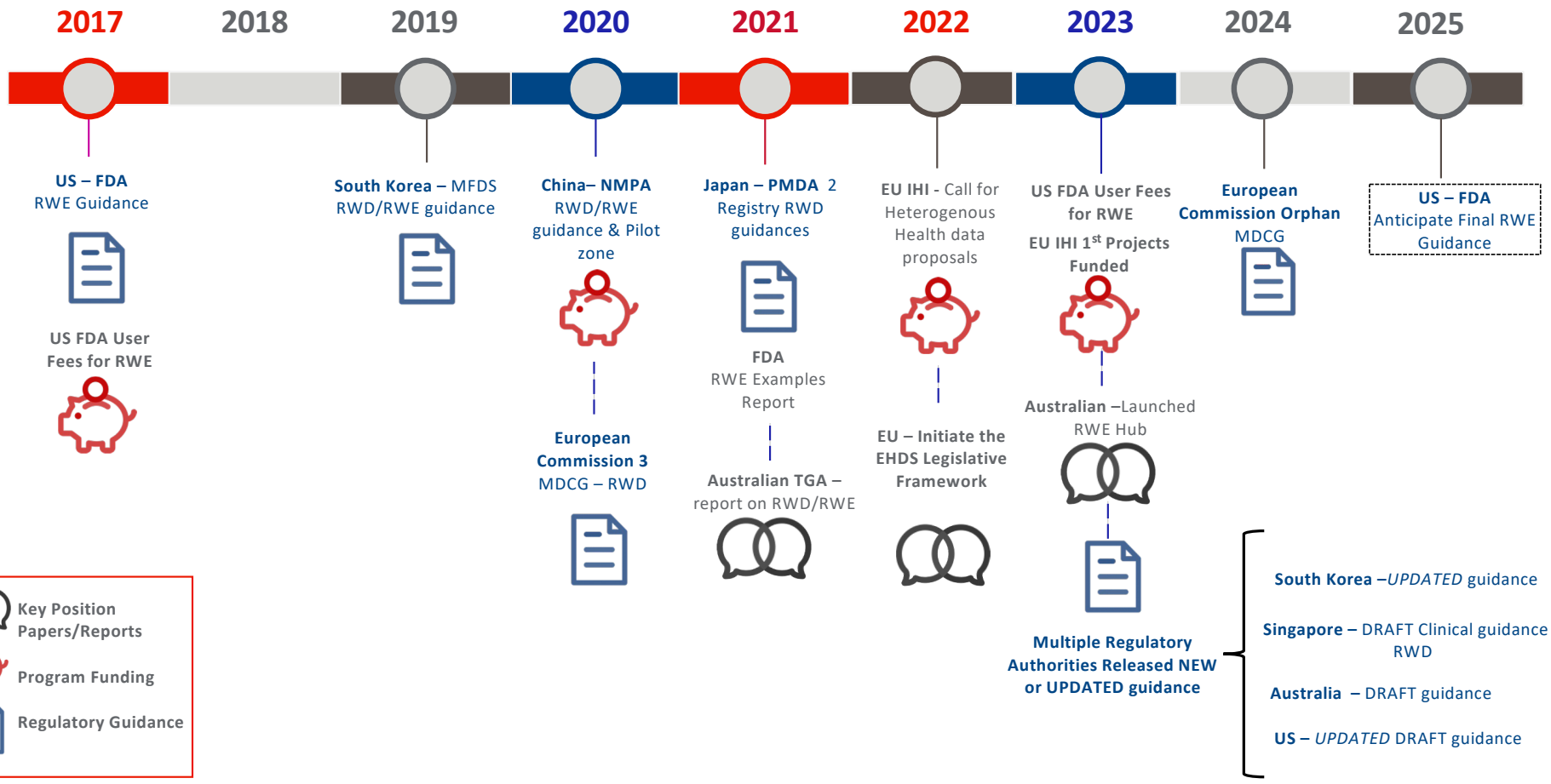


*"**Real-World Evidence (RWE)** is the clinical evidence regarding the usage, and potential benefits or risks, of a medical product derived from analysis of RWD."*

Real-World Data Sources



Medical Device RWE Activities & Guidances



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US FDA Recent Policy Guidance Overview

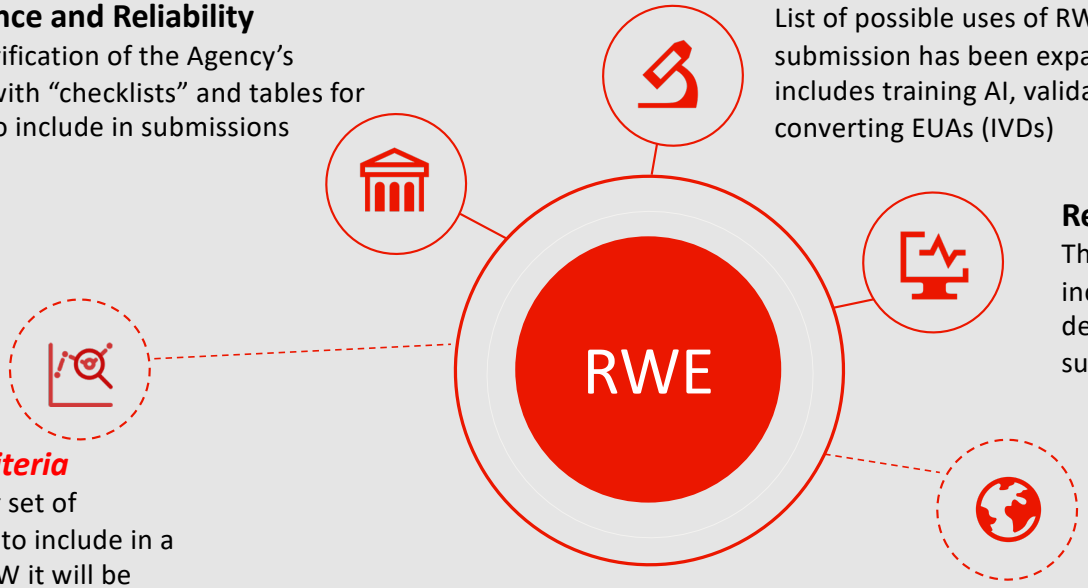
FDA CDRH RWE Guidance

2023 DRAFT

Main Take-Aways: Generally, similar components as 2017 version. Updates include expanded details on expectations AND more structured overview of the Agency's submission expectations.

Data Relevance and Reliability
Significant clarification of the Agency's expectations with "checklists" and tables for components to include in submissions

RWE Use Cases
List of possible uses of RWE in a regulatory submission has been expanded and now includes training AI, validating COAs and converting EUAs (IVDs)



Real-World Data Sources
The examples of RWD has been expanded to include patient-generated health data (PGHD), device-generated data and public health surveillance data

RWE Assessment Criteria
Draft provides a clearer set of expectations for WHAT to include in a submission but not HOW it will be assessed by the Agency

Good Research Practices
Draft includes reference to Good Clinical Practice (GCP) and our policy shaping will focus on supporting the development of consensus on good RWE research practices.

Source: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/draft-use-real-world-evidence-support-regulatory-decision-making-medical-devices

Common Considerations

Design Considerations for Pivotal Clinical Investigations for Medical Devices

Guidance for Industry, Clinical Investigators, Institutional Review Boards and Food and Drug Administration Staff

Document issued on: November 7, 2013

The draft of this document was issued on August 15, 2011.

For questions regarding this document that relate to devices regulated by CDRH, contact Gregory Campbell, PhD at (301) 796-5750 or by email at greg.campbell@fda.hhs.gov, if desired.

For questions regarding this document that relate to devices regulated by CBER, contact Stephen Ripley at 301-827-6210.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

Contains Nonbinding Recommendations

Draft – Not for Implementation

Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices

Draft Guidance for Industry and Food and Drug Administration Staff

DRAFT GUIDANCE

This draft guidance document is being distributed for comment purposes only.

Document issued on December 19, 2023.

You should submit comments and suggestions regarding this draft document within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff, Food and Drug Administration, 5630 Fishers Lane, Room 1061, (HFA-305), Rockville, MD 20857-1740. Identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions about this document regarding CDRH-regulated devices, contact the Office of Clinical Evidence and Analysis at CDRHClinicalEvidence@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010, or by email at ocod@fda.hhs.gov.

When final, this guidance will supersede "Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices," issued August 2017.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

More guidance on submission components & expectations

Appendices

- **Table of Recommended RWD Relevance Elements for Submission of RWE**
- **Table of Recommended RWD Reliability Elements for Submission of RWE**
- **Examples Where RWE is Used**
  - New or Expanded Indications for Use
  - 522 Submissions
  - Control group
  - Supplementary Data
  - RWE Obtained from Use of EUA device

Table 1- Recommended RWD Relevance Elements for Submission of RWE

Item (Linked to Section V.)	**Information for Sponsors to Document** (e.g., to make available for inspection)	**Information for Sponsors to Provide to FDA in Submission**	**Recommended Location in FDA Submission**
Determine RWD source contains sufficient detail to capture data elements and address the study question	x (detailed)	x (rationale)	Protocol (rationale for study question and data element definitions)
Assess longitudinality of data source		x	Protocol
Assess continuity of care in data source		x	Protocol and report
Ensure reasonable time between data collection and release for research		x	Protocol and report
Consider changes in clinical practice/guidelines over time	x	x	Protocol
Assess timing of availability of any new (i.e., updated) data after initial data availability		x	Protocol and report
Assess whether and how data from different sources can be obtained and integrated, given the potential for heterogeneity in population characteristics, clinical practices, and coding across data sources		x	Protocol
If done, use of a predefined linkage methodology that is scientifically valid and accounts for differences in coding and reporting across sources	x (detailed)	x (high level)	Protocol

Role of Public-Private Partnerships to Advance Regulatory Science and Policy

innovative health initiative

IHI is a partnership for health research and innovation between the EU and Europe's life science industries.

COCIR Advancing Healthcare

efpia

EuropaBio The European Association for Bioindustries

MedTech Europe from diagnosis to cure

Vaccines Europe

Co-funded by the European Union

IHI core goals are to:

- translate health research and innovation into tangible benefits for patients and society, and
- ensure that Europe remains at the cutting edge of interdisciplinary, sustainable, patient-centric health research.

Integration of Heterogeneous Data and Evidence towards Regulatory & HTA Accetance (IDERHA)

academia & research & public

LUDWIG BOLTZMANN INSTITUTE Digital Health and Prevention

LÆGEMIDDELSTYRELSEN DANISH MEDICINES AGENCY

SERVICIO ANDALUZ DE SALUD Consejería de Salud y Consumo

FH University of Applied Sciences TECHNIKUM WIEN

JE FACTUM

EPF European Patients Forum

lygature pioneering medicine. together.

iHD The European Institute for Innovation through Health Data

Fraunhofer

IIS La Fe Health Research Institute

ITTM Information Technology for Translational Medicine

MSB Medical School Berlin Hochschule für Gesundheit und Medizin

ASKLEPIOS

uni.lu | LCSB

VTT

MRI Klinikum rechts der Isar Technische Universität München | TUM

MEDICAL UNIVERSITY OF VIENNA

SERVIZO GALEGO DE SAÚDE

industry & SM

Johnson&Johnson

Johnson&Johnson MedTech

PHILIPS

Takeda

sanofi

labcorp

Roche

associated

ERS EUROPEAN RESPIRATORY SOCIETY every breath counts

hygiaso

LUCE Lung Cancer Europe

NICE National Institute for Health and Care Excellence

affiliated

Junta de Andalucía Consejería de Salud y Consumo

IDERHA integrating health data

IDERHA Vision

Integration of heterogenous Data and Evidence towards Regulatory & HTA Acceptance

Build an open, disease agnostic and federated data space which enables connectivity, access, use and reuse of digital health data

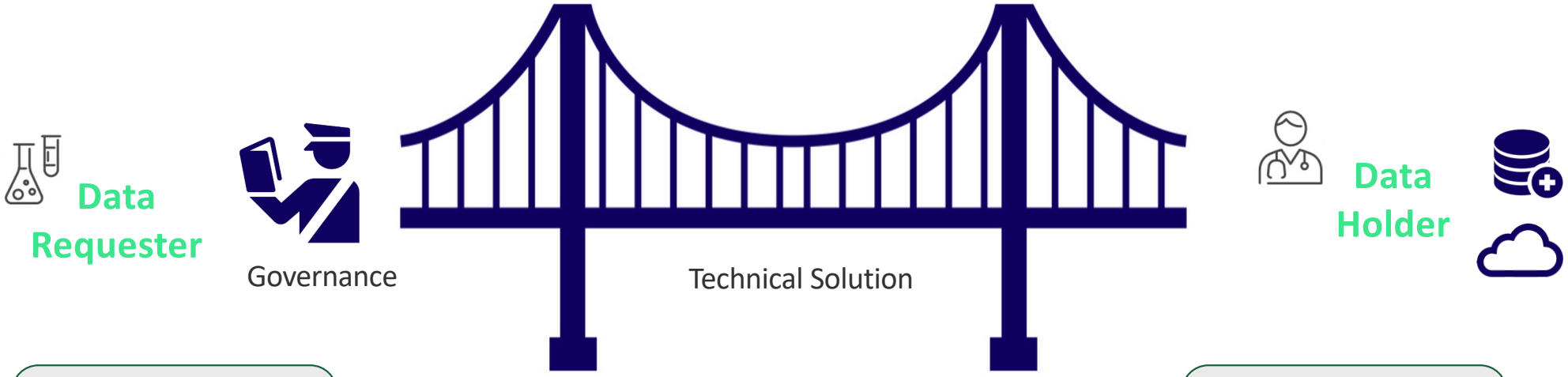
Develop consensus policy recommendations on

1) health data access

2) heterogeneous health research (e.g., RWE) for regulatory & HTA decision-making

IDERHA Secure Data Access Infrastructure Principles

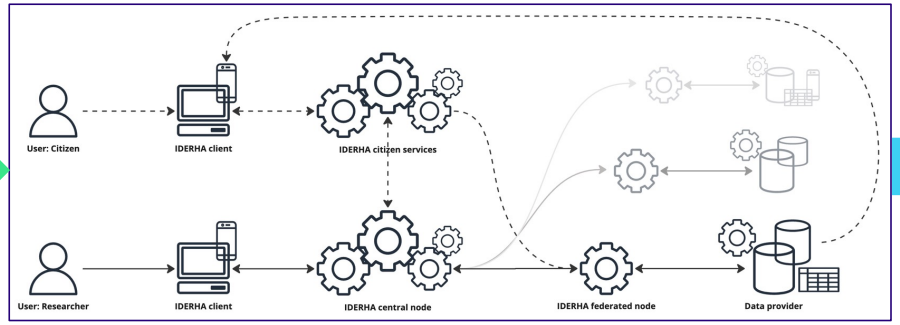
Based on standards, enabling interoperability & easy re-use of existing solutions incl. FAIR, GA4GH* & IDSA**



*GA4GH: Global Alliance for Genomics and Health (GA4GH) sets standards and frames policies to expand genomic data use https://www.ga4gh.org/
**https://internationaldataspaces.org/

IDERHA integrating health data

Policy Barriers, Challenges and Evolution



Clear governance to enable Health Data Access & Sharing

Criteria for determining the Acceptability of RWE for Regulatory & HTA Decision-making

Ongoing Policy Changes
(EHDS & AI regulations)

IDERHA
integrating health data

Funded by innovative health initiative

Policy Goal

Provide well-researched and consensus-driven recommendations to ***Inform*** and ***Accelerate*** harmonized policy development

Landscape of Current RWD/RWE Policies

Define Policy Priorities

Engage with Key Stakeholders

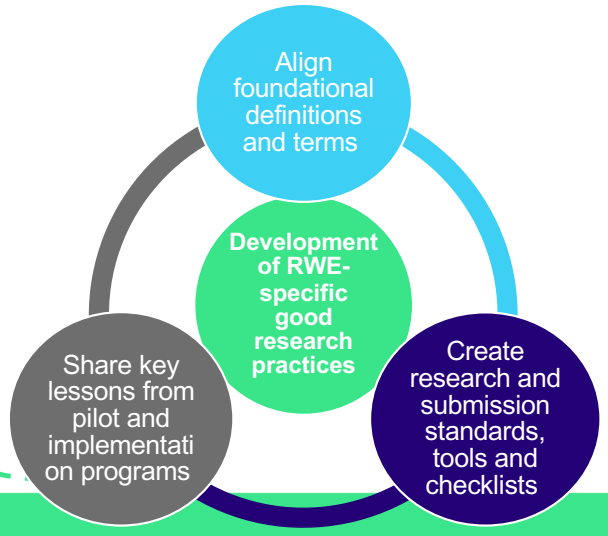
Develop Recommendations

Evolving Perspectives

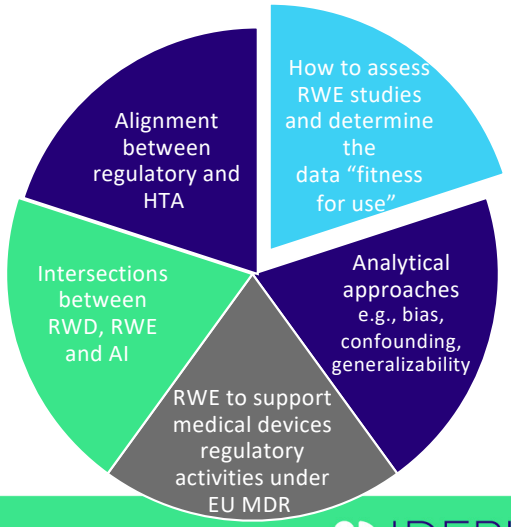
- **Potential benefits of RWE**
- **Policies and requirements reflect concerns about the potential for poor quality research and misuse**

Finding: To build trust, policy & programmatic changes needed to ensure high-quality and transparent research.

Emerging Best Practices



Key Policy Gaps



https://www.iderha.org/

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integrating health data