GDD Regulatory & Development Policy

RWD for regulatory decisions

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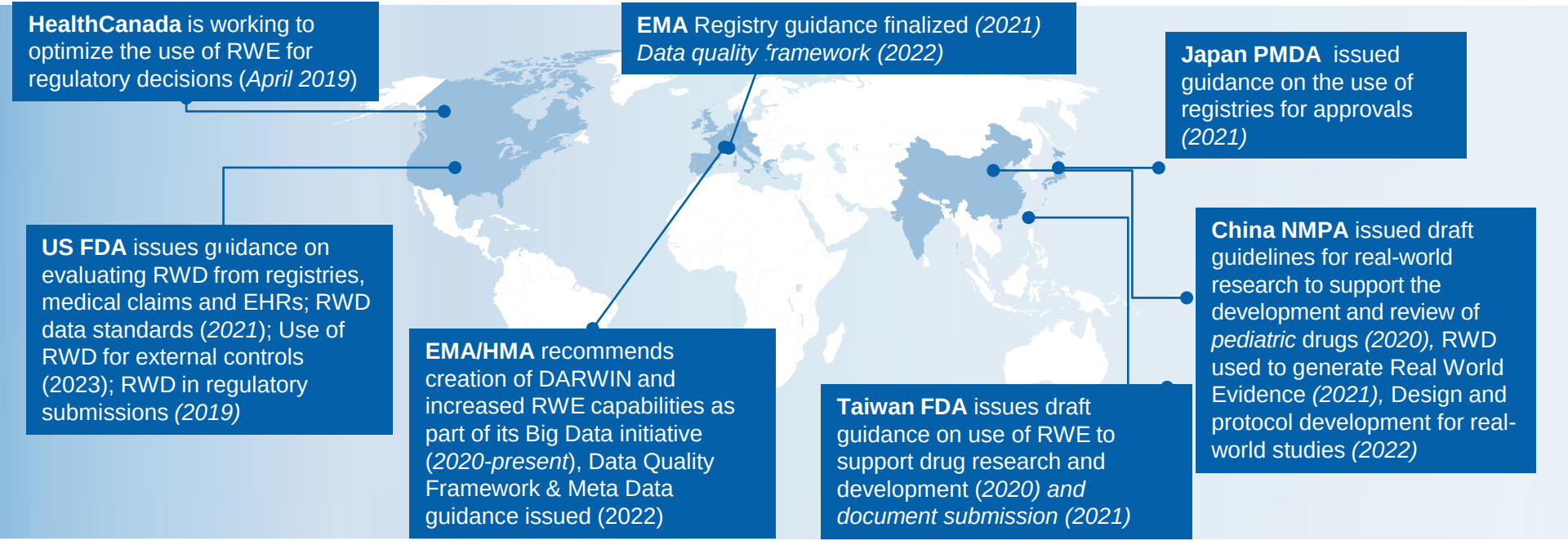
RWD/E guidance & frameworks

Health authorities have issued frameworks and guidance on the use of RWD/E for medical product development and regulatory decision making



EMA, European Medicines Association; US FDA, Food and Drug Administration; TFDA, Taiwan Food and Drug Administration; HA, health authority; HMA, Heads of Medicines Agencies; RWD, real world data; RWE, real world evidence.

Expanded possibilities for RWD/E

The use of RWE for regulatory decisions is not new, but interest in potential opportunities to expand its use beyond safety is being driven by:

Greater availability of electronic healthcare information

Capability to analyze large volumes of data

Ability to link patient data across sources and care settings

Appreciation of the limitations of traditional trials

Definitions*of 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

- electronic health records (EHRs)
- medical claims data
- product and disease registries
- patient-generated data, including in-home settings
- data gathered from other sources, such as mobile devices, that can inform on health status

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

Generated using different study designs, including but not limited to randomized trials (e.g., large simple trials, pragmatic trials), externally controlled trials, and observational studies

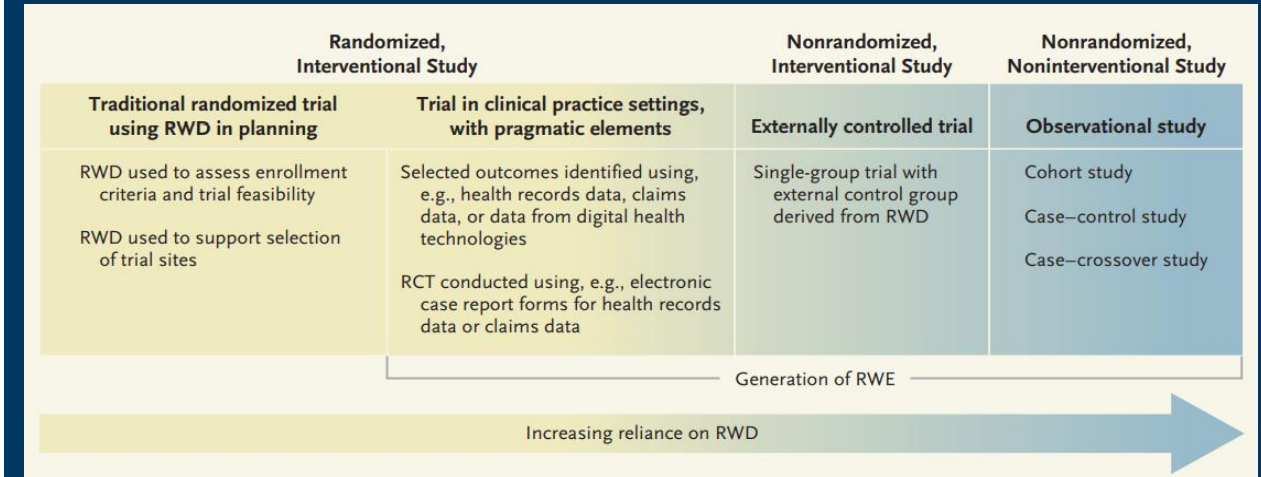
Adapted from FDA presentation May 12, 2021
To be aligned as per icmra_statement_on_rwe.pdf

Generating RWE

RWD studies exist on a continuum

A variety of study types may generate fit-for-purpose RWE

Reliance on RWD in representative types of study design[1]



RCT, randomized, controlled trial.

1.Concato J and Corrigan-Curay J. *N Engl J Med* 2022;386:1680–2.

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Considerations for regulatory decisions

Important considerations when using RWD/E for regulatory purposes

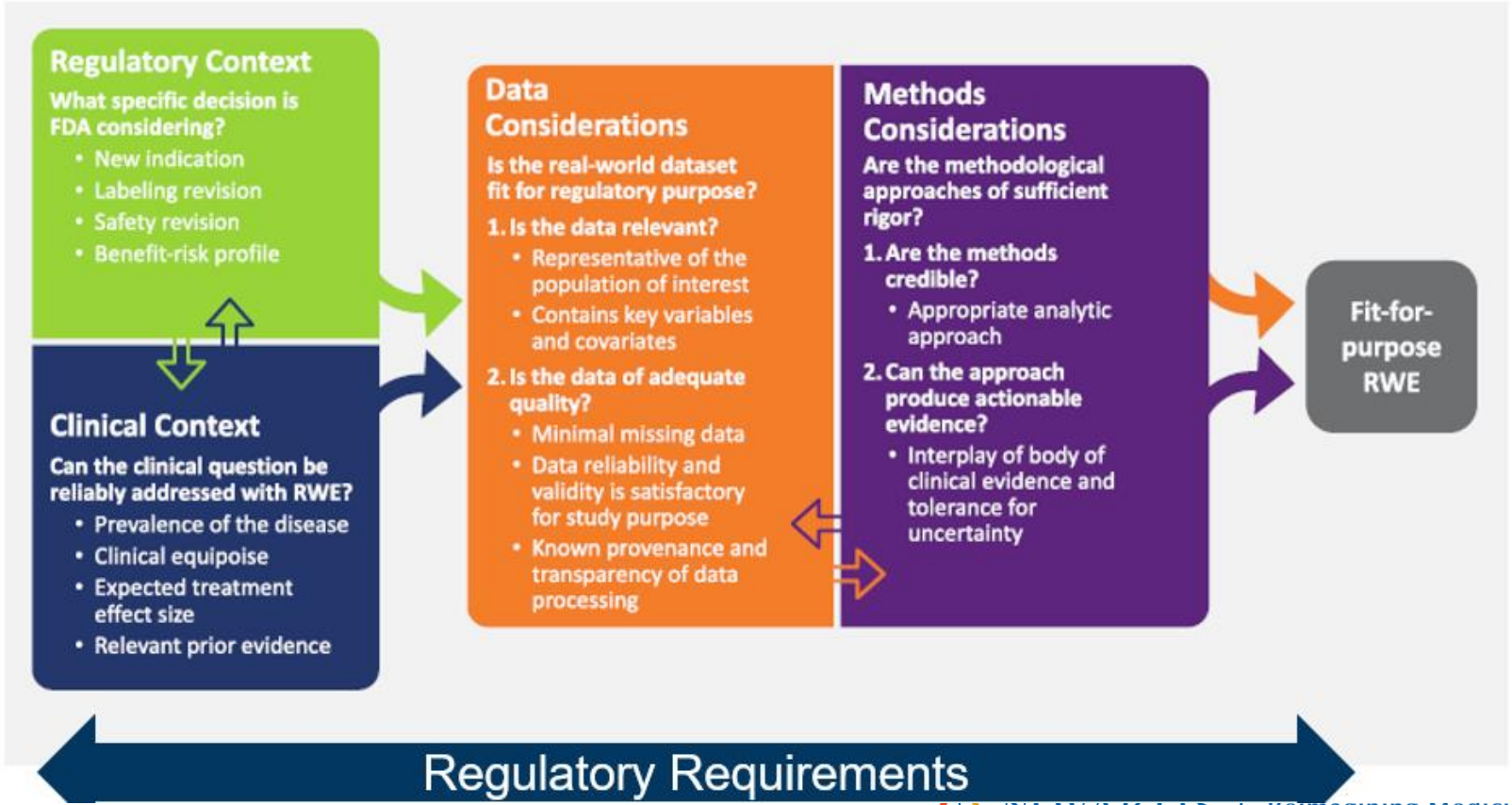
1. Are the **RWD** "fit for use" in regulatory decision-making?

2. Can the study design and methods used to generate **RWE** provide adequate and interpretable scientific evidence to support a specific regulatory decision?

3. Does the approach used in a particular case meet applicable regulatory requirements, including:
   - Requirements for study monitoring
   - Pre-specification of protocols/SAPs
   - Established standards for data submission

MHRA guidance on the use of real-world data in clinical studies to support regulatory decisions - GOV.UK (www.gov.uk); Framework for FDA's real-world evidence program. Available from: https://www.fda.gov/media/120060/download; Guideline on registry-based studies (europa.eu)

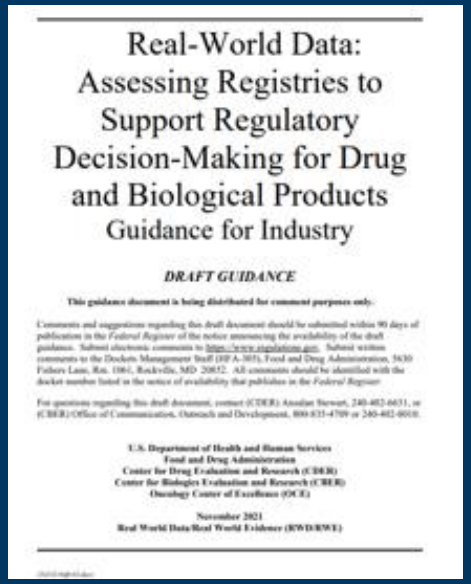
Framework for "decision grade" RWE



Adapted from: Characterizing RWD Quality and Relevancy for Regulatory Purposes (duke.edu)

Regulatory guidance: where are we now?

Several draft regulatory guidance documents on RWD quality exists, with more to come



Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

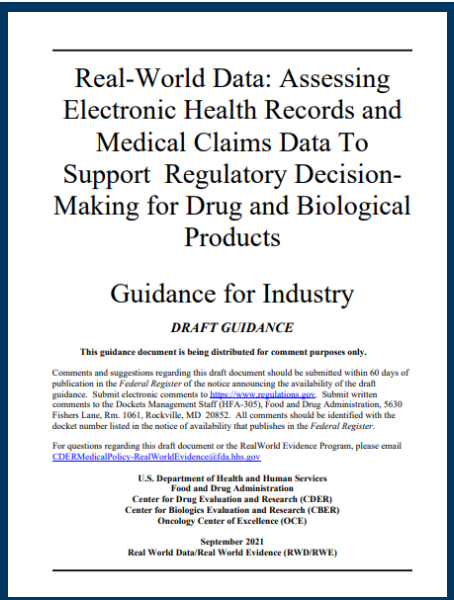
This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 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 (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Anuliar Stewart, 240-402-6431, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

November 2021
Real World Data/Real World Evidence (RWD/RWE)



Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

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For questions regarding this draft document or the RealWorld Evidence Program, please email CDERMedicalPolicy-RealWorldEvidence@fda.hhs.gov

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

September 2021
Real World Data/Real World Evidence (RWD/RWE)

HMA
Heads of Medicines Agencies

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

30 September 2022
Data Analytics and Methods Task Force
European Medicines Agency

Data Quality Framework for EU medicines regulation

Start of public consultation	10 October 2022
End of consultation	18 November 2022

Comments should be provided using this template. The completed comments form should be sent to dataqualityframework@ema.europa.eu

Keywords	Data sources, studies, metadata, study protocol, study report, data flows, data management, vocabulary, glossary, use cases, population

DRAFT

data-quality-framework-eu-medicines-regulation_en.pdf (europa.eu)
Use of Electronic Health Record Data in Clinical Investigations Guidance for Industry (fda.gov)
Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products

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ICMRA's collaboration pledge on RWD/E

International Coalition of Medicines Regulatory Authorities (ICMRA) pledged to work towards alignment on four key areas related to regulatory uses of RWD:

- **Harmonization of RWD and RWE terminologies**
  - Common definitions for RWD/E
- **Convergence on RWD and RWE guidance and best practices**
  - Common quality principles; protocol templates; alignment on suitable use cases for RWD/E in regulatory decision making
- **Readiness**
  - Collaboration on RWD studies to address public heath
- **Transparency**
  - Systematic registration of RWD studies in public registries

Implementation will happen through collaboration via ICH, meetings between interested regulators and standard setting bodies

icmra_statement_on_rwe.pdf

Applying RWD regulatory frameworks

During the workshop, keep the regulatory purpose and frameworks in mind

- What does data quality mean?
- How can we assess and demonstrate that RWD is fit-for-purpose?
- Are there areas of regulatory uncertainty need to be addressed?
- What practical tools are needed in the future?

Questions

Thank you

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