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Canada

Progress on Convergence with International Partners on Real World Data

EU Big Data Stakeholder Forum

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Background

- Growing availability of Real-World Data (RWD) for drug safety and efficacy assessment.

- Evolving focus on RWD/RWE in the therapeutic product life cycle by regulators, health technology assessors, industry, and stakeholders.

- Various initiatives addressing challenges RWD/RWE.
  - In Canada, the Guidance for Reporting RWE and accompanying Position Statement
  - In the EU, the HMA/EMA Big Data initiative
  - In the US, the RWE framework

- But challenges remain with respect to definitions, data sources, fitness, quality, methodological challenges, data sharing and access.

International Collaboration on Convergence

- To address challenges in the RWD/RWE space, regulators have been working together to advance a number of initiatives:

  - **International Coalition of Medicines Regulatory Authorities (ICMRA)** COVID-19 Real World Evidence (RWE) and Observational Studies Working Group (WG).

  - **International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) M14** - "*General principles on the plan, design, and analysis of pharmacoepidemiological studies that utilize real-world data for safety assessment of medicines.*"

  - **ICH Reflection Paper** on "*International harmonisation of real-world evidence terminology, and convergence of general principles regarding planning and reporting of studies using real-world data, with a focus on effectiveness of medicines.*"

COVID-19 – An Opportunity for Collaboration

- The **ICMRA COVID-19 RWE and Observational Studies WG**, co-chaired by the **EMA and HC**, was created in April 2020.

- Regulators from more than **20 member agencies** have participated to:
  - Discuss observational studies to characterise COVID-19 disease, links between clinical outcomes and concomitant medication use, and surveillance and the safety and effectiveness of vaccines and treatments
  - Explore the feasibility of collaboration on research projects
  - Exchange information on vaccine pharmacovigilance and public communications
  - Provide experiences and lessons learned
  - Discuss path forward

ICMRA COVID-19 RWE WG Achievements

- Demonstrated value respond to a public health emergency through international collaborations on observational studies, vaccine pharmacovigilance, and public communications.

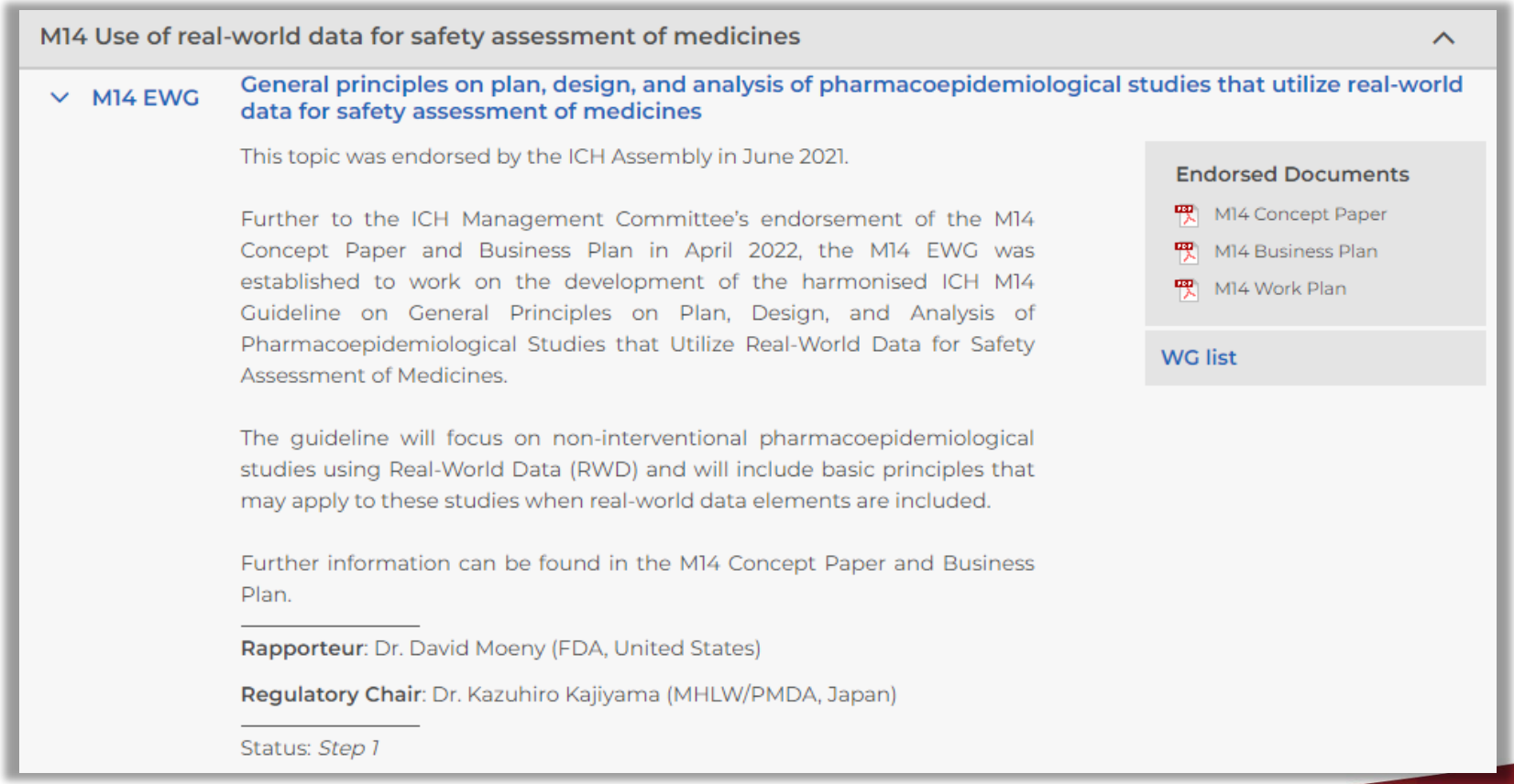
- Published collaborative studies on **steroid use, coagulopathy, case definitions, and COVID-19 treatments in pregnancy**.

- Statements on **international collaboration** and **vaccine safety**.
  - ICMRA statement on international collaboration to enable RWE for regulatory decision-making
  - ICMRA/WHO statement for healthcare professionals on safety and effectiveness of COVID-19 vaccines

- **Member feedback initiatives** on lessons learned.
  - Survey among members on future collaboration insights
  - Survey among members on lessons from past activities and strategic options moving forward

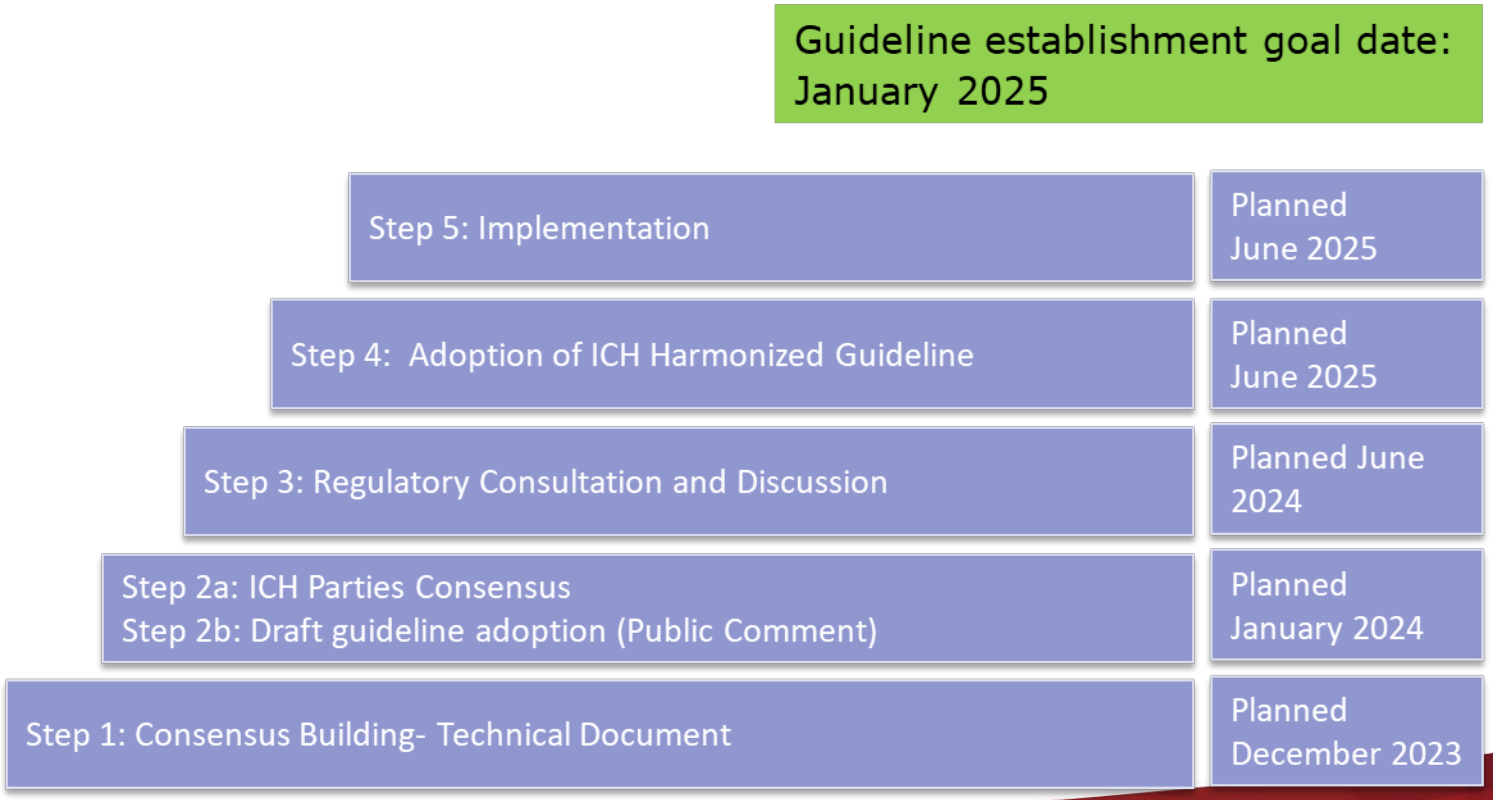
ICH M14 Guideline

- Establishing a guideline for **post-approval safety studies using RWD**.
- Focus on drugs, vaccines, and biologics.



ICH M14 – Current Status

- First draft under internal review (June 2023) **– currently reviewing member comments (N = 615).**
- Anticipation to proceed to Step 2 by Dec 2023.



ICH RWE Reflection Paper

International harmonisation of real-world evidence (RWE) terminology and convergence of general principles regarding planning and reporting of studies using real-world data (RWD), with a focus on effectiveness of medicines

- **EMA, FDA,** and **HC** worked together to develop a Reflection Paper (RP) focusing on key areas identified through the June 2022 ICMRA Regulatory Workshop and July 2022 ICMRA Statement on international collaboration.
- Represents the initial step of an incremental approach towards **harmonisation of regulatory RWE guidance**.

- **Public consultation** ending September 30, 2023: received 151 comments from 16 organizations, with overall support for the two proposed guidelines.

- EMA/FDA/HC reviewing comments, with finalised RP targeted for adoption by the ICH Assembly in June 2024.

ICH RWE RP Stepwise Approach

2 Proposed Guidelines

	Topic	Objective	Deliverables	Tentative timeframe
1.	**RWD/RWE terminology, metadata, and assessment principles**	• Promote a common understanding of the types and scope of RWD/RWE • Guide the discoverability, identification, and description of RWD • Inform the assessment of RWD/RWE for regulatory purposes	• Common operational definitions of RWD and RWE, with clear scope, breadth of potential RWD sources, and level of granularity (e.g., pertaining to RCTs and non-interventional studies)[1] • Core list and use of metadata • General principles for assessment of RWD/RWE	Submit new ICH topic proposal in Dec 2023 or Dec 2024
2.	**RWD/RWE protocol & report format, and study transparency**	• Agree on common principles regarding formats for RWD/RWE protocols and reports of study results submitted to regulators • Promote transparency by encouraging registration of study protocols and study reports in publicly available registries	• Principles for structure and content of protocols and reports (for medicines developers) • Recommended "best practices" for registration of study protocols/results	Initiate work after the first guideline reaches *Step 4* of the ICH Procedure

[1]: Reference to the ICH M14 glossary will be made during development of the Concept Paper

Concluding Thoughts

Global Progress in RWD/RWE:

- Growing availability of RWD and evolving focus throughout the product life cycle.
- Numerous initiatives underway to address challenges.

International Collaboration Highlights:

- ICMRA COVID-19 RWE Working Group and collaborative achievements during the pandemic.
- ICH M14 Guideline development for post-approval safety studies using RWD.

Current Milestones:

- ICH M14: First draft under review, proceeding to Step 2 in Dec 2023.
- ICH RWE Reflection Paper: Public consultation completed; adoption of finalised RP targeted for June 2024.

Future Landscape:

- Harmonization/convergence on global RWE terminology and principles.
- Leveraging insights from collaborative studies.

Thank you