RWE Working Group

Assessing Quality and Usability of Real-World Data Sources



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Introduction

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- **PHUSE RWE Project started in 2024**
  - Quality and Reusability of Real-World Data Sources
- **Presentations, Posters, and Workshop at PHUSE Conferences**
- **White Paper focussed on**
  - Regulatory landscape – what are the needs
  - Assessing Quality – what are the issues
  - Technical Challenges – how can we do it
- **Including RFI to assess quality of RWD sources**



Regulatory Landscape

Definition

- **Real-World Data**
  - Data relating to patient health status and/or the delivery of healthcare routinely collected from a variety of sources.
  - Sources
    - Electronic Health Records (EHR)
    - Insurance claims
    - Patient Generated Health Data (PGHD)
    - Registries
  - A real-life perspective on healthcare outcomes

Regulatory guidances included

- **Considerations for the Use of RWD and RWE to Support Regulatory Decision-Making for Drug and Biological Products (FDA, August 2023)**
- **Data Quality Framework for EU Medicines Regulation (EMA, October 2023)**
- **Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products (FDA, December 2023)**
- **Real-World Data: Assessing Electronic Health Records and Medical Claims Data to Support Regulatory DecisionMaking for Drug and Biological Products (FDA, July 2024) (2): Outlines strategies for data selection, validation, and handling missing data to meet reliability requirements.**



RWD Quality Principles & Challenges

- **Fit for use**
  - Reliability
  - Relevance
  - Integrity

- **Challenges**
  - Avoid Bias
  - Study Design
  - Informed Consent
  - Data Governance



Regulatory Engagement

- **Early engagement**
  - Reduces the risk of misalignment
  - Addresses potential challenges
- **In study design phase**
- **Discussion points**
  - Data selection strategies
  - Feasibility of linking data sources
  - Data analysis strategies
  - Ensuring data traceability



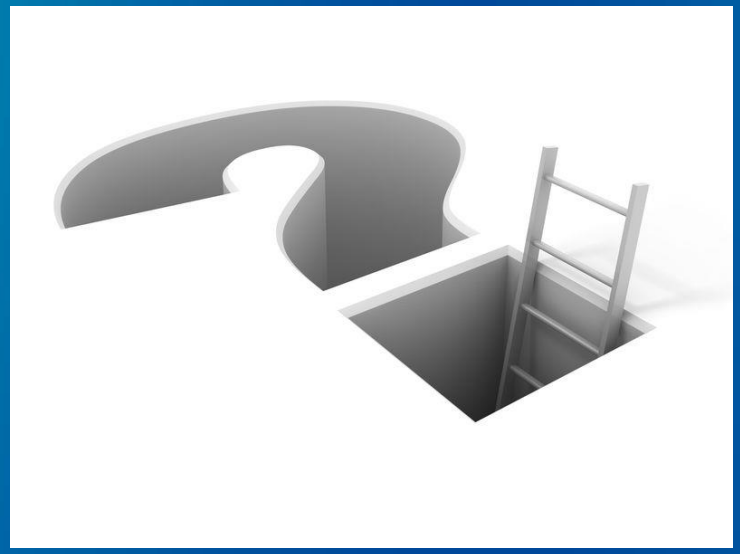
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Assessing Data Source Feasibility

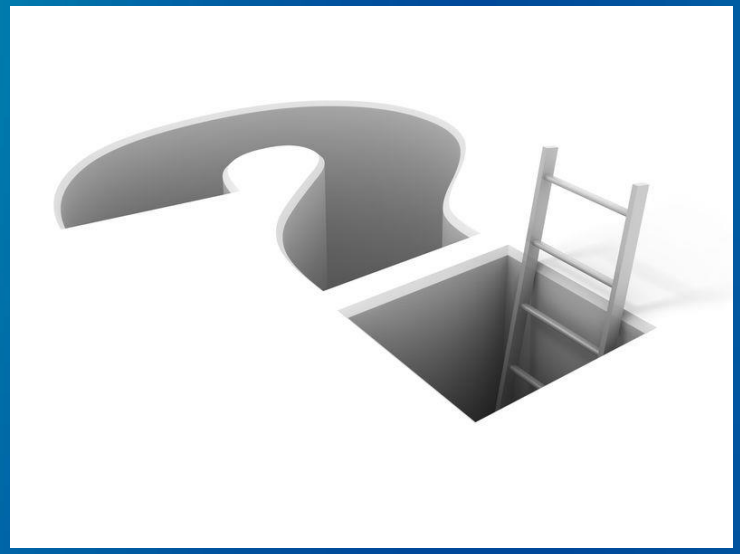
Feasibility Challenges

- **Continuity of Coverage**
- **Reliability and Integrity**
- **Accuracy and Representativeness**
- **Data standards**
- **Required Data Elements**
- **Regional Differences**
- **Sample Data**
- **Time Stamping**



Feasibility Assessments

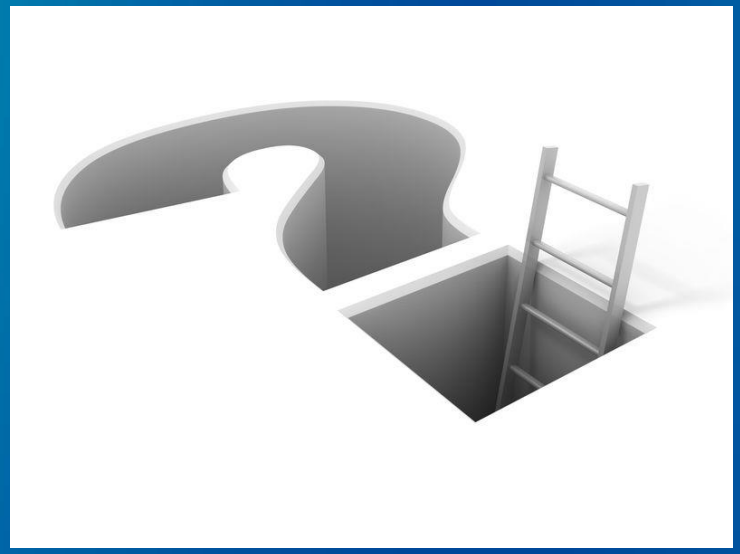
- **SPIFD2:**
  - Minimal criteria for valid operationalisation in RWD
- **Data Provider Request For Information (DPRFI)**
  - Tailored to the study needs
  - Covers all fit for purpose aspects with detailed questions
- **Stepwise contracting approach**
  - Fit for purpose assessments are often tedious and time consuming



DPRFI

- **Study Specific requirements**
  - Eligibility criteria
  - Relevant Data Elements (required or preferred)

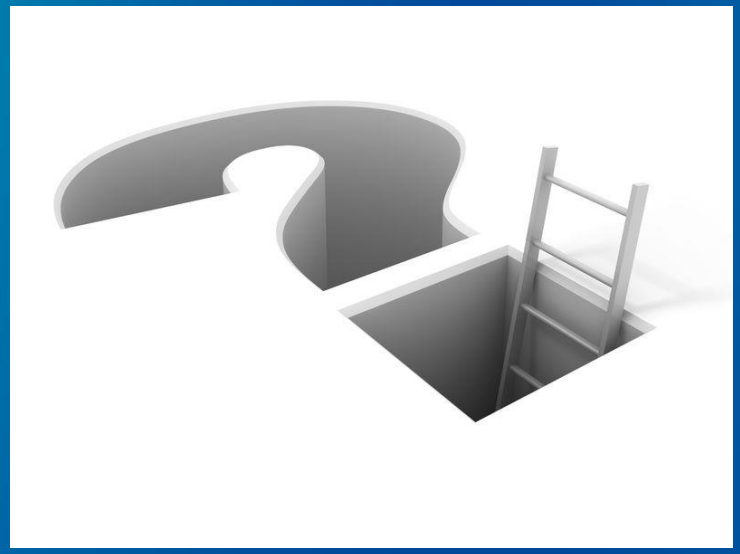
- **Data source outline**
  - Origin
  - Governance
  - Methods
  - Standards



DPRFI

- **Data Quality and Usability**
  - Completeness and Coverage
  - Provenance and Traceability
  - Quality and Accuracy

- **Data Services and Delivery**
  - Granularity
  - Experience
  - Sample Data
  - Costs



Technical Reliability

Technical Challenges

- **Data Anonymisation**
  - Encryption
  - Tokenisation
- **Data Lineage**
- **Data Abstraction from unstructured data**
  - NLP processes



NLP Process

- **Challenges**
  - Data Versioning
  - Data Lineage Tracking
  - Model Versioning
  - Pipeline Modularity
  - Experiment Logging
  - Audit Logging
  - Explainability Integration
  - Intermediate Output Storage
  - Data and Model Monitoring
  - Documentation and Governance



Next Steps

-Download White Paper

-NEW Project: RWD Data Lineage

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THANK YOU!