Real-World Evidence for Diagnostics: Data Collection and Quality

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What are RWD and where do they come from?

Real world *data* are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources. RWD can come from a number of sources, for example:

- Electronic health records (EHRs)
- Claims and billing activities
- Product and disease registries
- Patient-generated data including in home-use settings
- Data gathered from other sources that can inform on health status, such as mobile devices

What is RWE?

Real world *evidence* is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD.

https://www.fda.gov/scienceresearch/specialtopics/realworldevidence/default.htm
What Constitutes Quality in Registries & RWE?

✓ A full view of the patient journey-settings, types of data
✓ Representativeness
✓ Standard Outcomes
✓ Missing Data
✓ Completeness of data
✓ Reliability of Imputations
✓ Timeliness

Relevance

Characteristics

- **Sufficient detail to capture use of the device, exposures, and the outcomes of interest in the appropriate population**

- **Data elements available are capable of addressing the specified question**

- **RWD and RWE are interpretable**

*Factors in considering interpretability*

- Whether representative of real-world use
- Whether regional, national, and/or international
- The overall percentage of patient exposure captured in RWD source
- Validation protocols that are used to evaluate how well RWD source reflects patient population’s experience
- Whether appropriate to address regulatory question and can be accomplished in a timely manner
- Capture specific device identification
- Whether RWD source captures medical history, pre-existing conditions and follow-up
- Whether sufficient data to adjust for confounding
- Whether any linkages performed are scientifically appropriate
- The RWD reporting schedule
- Prior use of the RWD source
- Whether data elements collected are sufficient for assessing outcomes (including adjudication, if necessary)
- Whether supplemental data source are available and sufficient to provide any missing information or evidence

Source: Use of real-world data to support regulatory decision-making for medical devices, August 31, 2017, FDA
Reliability

Primary Factors

• How the data were collected (data accrual)
  • Preparedness of sites for complete and accurate collection of RWD (defined processes, site training, support, qualified personnel)
  • Whether common data capture form used
  • Whether a common definitional framework was used
  • Adherence to common temporal framework for key points
  • Timing of establishing study plan, protocol, analysis plan relative to collection or retrieval of RWD
  • Sources and technical methods used for data element capture (abstraction, POC, EHR integration, UDI, data records from devices, and linkage to claims data)
  • Whether patient selection criteria minimize bias and ensure representative real-world population
  • Timeliness of data entry, transmission and availability
  • Whether necessary and adequate patient protections were in place

• Whether people and processes in place during data collection and analysis provide adequate assurance that errors are minimized and data quality and integrity are sufficient (data assurance)

• Additionally, RWD analysis protocol should be prospectively defined

Data assurance-QC

• The quality of the data element population (e.g. whether abstracted from verifiable source to assess transcription errors or automatically populated through a data extraction algorithm)
• Adherence to source verification procedures and data collection and recording procedures for completeness and accuracy
• Data consistency across sites and over time

Source: Use of real-world data to support regulatory decision-making for medical devices, August 31, 2017, FDA
What’s Important in RWD/RWE in Diagnostics

Getting the Data that are Relevant

Ensuring it is Reliable and “Regulatory Grade”

Analyzing it for Decision-Making

Accrual

Processing and Quality Assurance

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How to Collect Quality RWD

Developed locations with HIT infrastructure and data availability

Less developed locations without HIT infrastructure or data availability

Leverage HIT systems

Implement systems
HIT Infrastructure and Data Availability

“Manual”

Familiar
Less likely to generate large numbers rapidly

“Automated”

Less Familiar
High scalability, more rapid, typically lower costs
Specialized Data Networks and Registries

Approvals, Training, Verification
Assessing Data Availability and Completeness

- Data Availability

% complete by data source for lab text x

Source: OM1 Internal Data
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Connecting EMRs and other systems
Critical Endpoints from Unstructured Data

Derive structured information from unstructured data in a validated and reliable manner.

Unstructured Data → Structured Data

Apply machine learning and MLP

Valid and reliable data points

With Performance Metrics for Each Output
Process c/w Current and Evolving Regs

Linkage from other sources

Highly Available, Traceable, Documented, Auditable
Traceability
When HIT systems and data are not available

- Mobile systems, centralized case reporting, etc.

FDA Unveils MyStudies App for Patient Data Collection
Emerging Requirements from FDA
In Summary:

RWD is fit for purpose
Study design used can provide adequate scientific evidence
Study conduct meets FDA regulatory requirements

Source: Framework for FDA’s Real-World Evidence Program
Questions

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