Unlocking the potential of real-world data through emerging data sources

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About me & my work

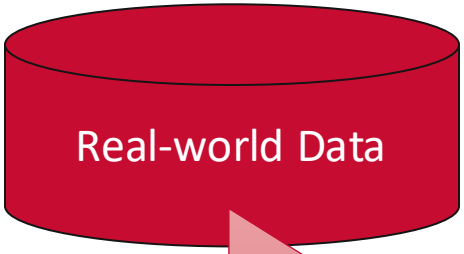
- Background in Biotechnology, Management & Data Science
- Data Manager/Data scientist for 7 years

- Family-owned medical device company
- Cochlear implants and other hearing implants

- Dedicated clinical research department
- Two full-time data managers
- Located in Innsbruck, Austria

From Data to Evidence

As defined by the FDA



Real-wo dence

"data relating to […] the delivery of health care routinely collected from a variety of sources"

"clinical evidence about the usage and potential benefits or risks of a medical product derived from analysis of RWD."

https://www.fda.gov/science esearch-center-drug-evaluation-and-research-real-world

Why Real World Data (RWD)?

- Regulatory demand (FDA, EMA, MDR etc.)
- Post-market surveillance
- Evidence for label extensions (e.g. Expandend indications for use)
- External control arms

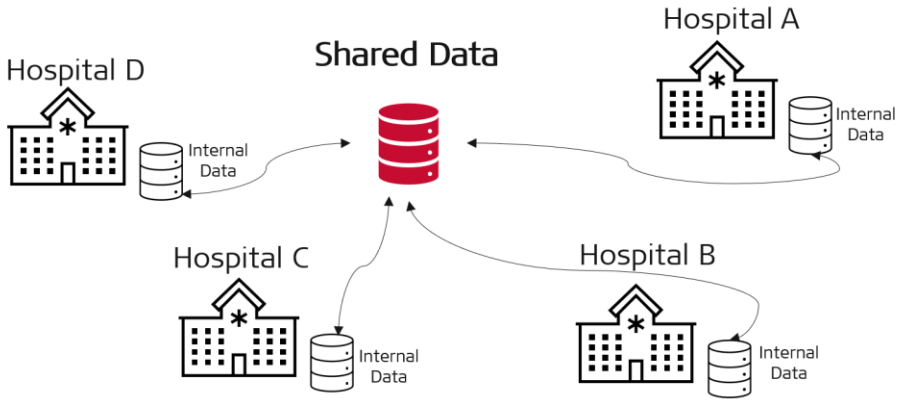
RWD closes the gap in RCTs

Flaws in RCT

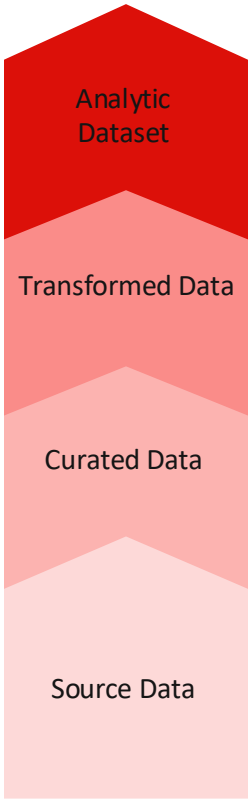
- Strict inclusion criteria
- Generalizibility
- Scaleability
- Long-term outcomes
- Can be unethical (if placebo is unethical)

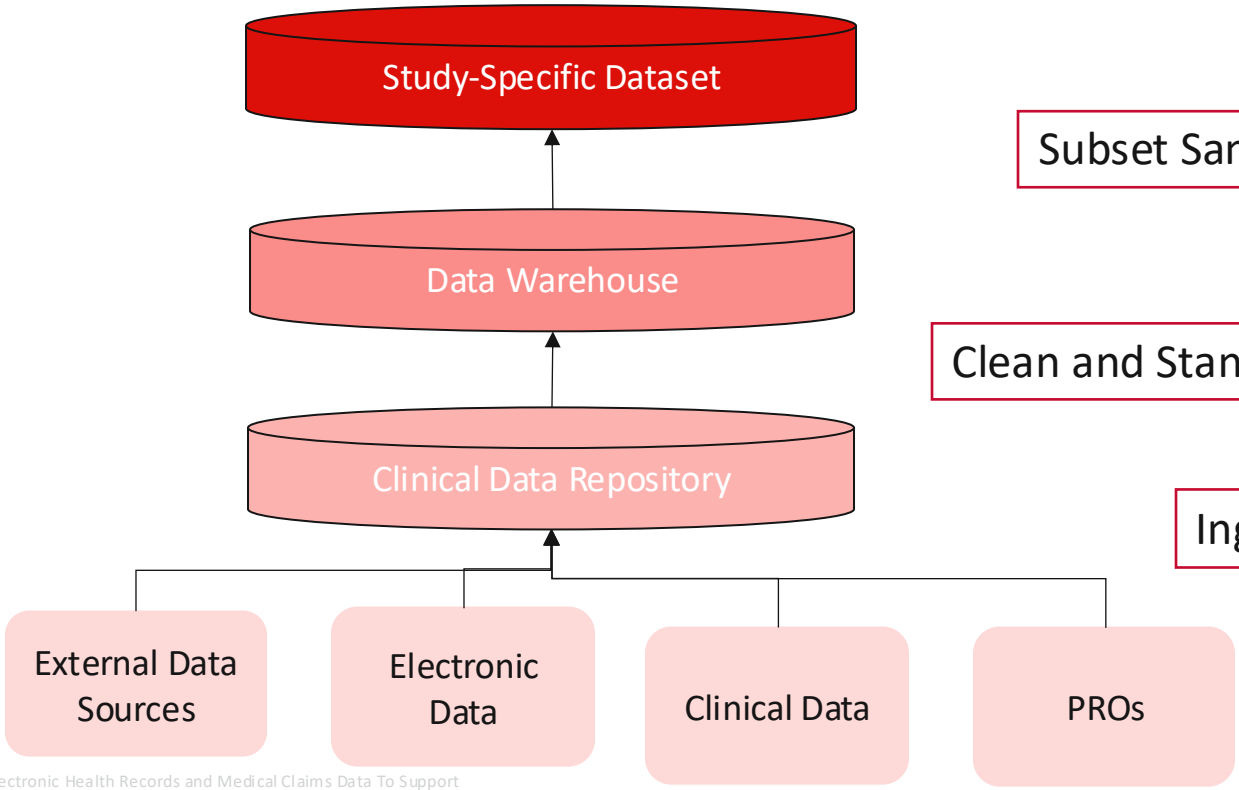
Data sources

- Non-interventional studies
- Registries
- Health records
- Medical claims databases
- User surveys
- Device data / apps



RWD data flows





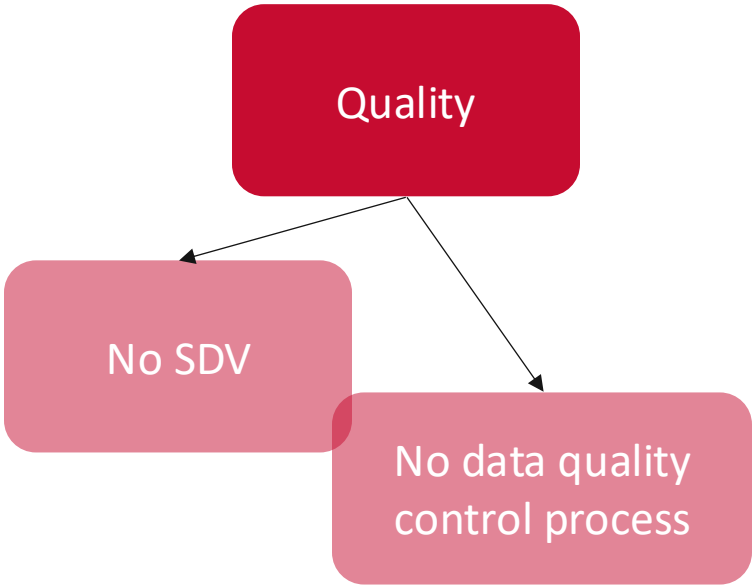
Subset Sample

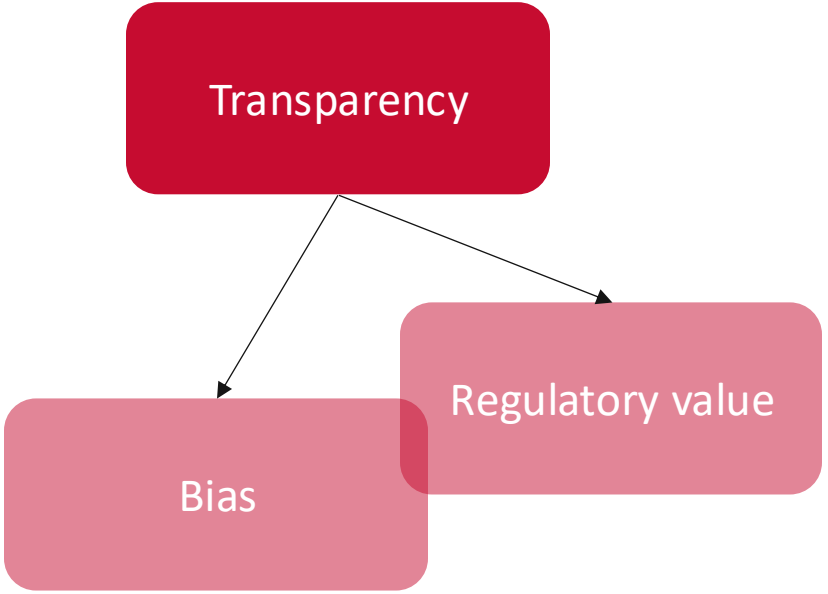
Clean and Standardize

Ingest

Adapted from: Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory DecisionMaking for Drug and Biological Products, FDA July 2024 (https://www.fda.gov/media/152503/download)

RWD Concerns





Ensuring regulatory-grade data quality

- FDA guidances on real-world data sources:

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

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

Data accrual

Not complete

- **Preparedness** of individual sites for **complete** and **accurate** collection
- Common data capture form & **data dictionary**
- **Sources** and **technical methods** used for data element capture
- whether patient selection and enrollment criteria **minimize bias** and ensure a **representative** real-world population
- whether necessary and adequate **patient protections** were in place

Data assurance – Quality control

Not complete

- the **quality** of data element population
- adherence to procedures to ensure **completeness** and **consistency**
- **data consistency** across sites and over time
- on-going **training** and use of **data dictionaries**
- site and **data monitoring practices**

How to achieve?

„Quality system approach“: Risk-based quality assurance & monitoring

- Validated tools
- Validated processes
- Site trainings
- Monitoring

Challenges for Data Managers

- New data collection tools
- Large data-sets
- Advanced skill-set: SQL, Python, PowerBI…
- Process design

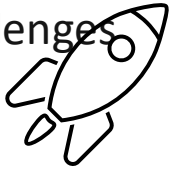
Opportunities and outlook

Opportunities

- Enhanced decision making
- Cost efficiency

Outlook

- Technological advancements
- More data -> more challenges



Summary

- **Regulatory demand** to use RWE
- **Variety** of data sources
- Data accrual must be **validated** and **verifiable**
- Quality **system approach**
- **Advanced** skill-set for data managers