Trends in RWD/RWE and Data Standardization

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RWE begins with the ability to completely understand patients like never before

- Patient Demographic and Socioeconomic Characteristics
- Clinical Parameters
- Outcomes Measures
- Lab Test Values
- Treatment Information
- Ordering/Administering Health Care Providers
- Provider Characteristics
- Payer Characteristics
- Payment and Reimbursement Schemes
- Patient Payment Levels
- Levels of Adherence

- Find Patients for Clinical Trials
- Diagnosis Patients with Rare Disease Earlier
- Evaluate Disease Progression
- Demonstrate Outcomes
- Understand Payer Dynamics and Patient Cost Burden
- Triggers that Predict the “Next Patient”
- Measure & Adapt Promotional Program Performance
Real World Evidence – Solution Map

Helping to generate the evidence you need

Applied Real-World Solutions
- RWE planning
- Data landscaping and sourcing
- Scientific value statements development
- RWE in clinical development
- RWE portal

Site-based Research
- Prospective observational studies
- Registries
- Patient reported outcomes
- Quality of life
- Enriched / Hybrid studies
- Comparative effectiveness
- Safety, surveillance, & regulatory studies

Secondary Research
- Clinical and economic value proposition
- Impact of out-of-pocket costs and utilization management
- Treatment patterns
- Natural history of disease
- Disease prevalence
- Burden of illness, unmet medical need
- Comparative effectiveness

Health Economic Modeling
- Health economic evaluations
- Global models and local adaptions
- Stakeholder-friendly presentations of models
- Budget impact models
- Indirect comparisons
- ICER/HTA submission
- Quality Measurement, QOL
Regulators are increasingly interested in how RWE may support regulatory decision-making

Despite challenges, traditional RCTs are the gold standard for drug evidence development
- Increasingly time and resource intensive to conduct
- Not broadly representative of the patients seen in actual clinical care
- May be unethical or infeasible to perform given small patient population sizes

RWD/RWE can be used to demonstrate medical product safety and effectiveness
- RWE reflects broader patient populations
- RCTs may not be generating evidence on endpoints that are truly useful to patients, providers, or payers
- RWE can fill remaining downstream evidence gaps

RWD/RWE can be used to improve the efficiency of clinical research
- Growing base of RWD from electronic health information infrastructure has enabled routine and increasingly robust collection of digital data at the point of patient care
Regulators are increasingly interested in how RWE may support regulatory decision-making

- **21st Century Cures Act**
  - Law in 2016

- **Draft guidance 2021**
  - 4 draft guidances released to satisfy mandate of Cures Act

- **Press Release**
  - Guidance on general principles on planning and designing pharmaco studies using RWD for safety assessment of medicine

- **DISHA**
  - Digital Information Security in Healthcare Act

- **DARWIN EU Initiative**
  - Started in 2022, pushing towards 2025 network strategy

- **RWE guidance**
  - Feb 2023 – Updated guidance
  - April 2021 – Use of RWD guidance

- **Registry Guidance**
  - March 2021 – guidance on utilization of registry for application and reliability

- **FEEDER-NET**
  - Government initiative to build federated network
Increased demand for data standardization and data quality

Motivated by vast sources of RWD, and the need to integrate and analyze quickly for key insights

**Key insights**

- Cohort Identification
- Clinical Characteristics
- Translational Research
- Prevalence & Incidence
- Drug Safety & Efficacy
- Comparative Effectiveness

**Data Extraction, Curation, Privacy and Security Technologies**

- Patients
- Physicians
- Clinical trials
- Conditions
- Social media
- Pharmacy
- Devices
- Lab/Biomarkers
- Research experience
- Claims
- Reference data
- Institutions/Health Systems
- Countries
- Mortality
- Prescriptions
- Registries
- Imaging
- PRO
- Genomics
- EMR
- Chart Review
- Checklist
- Research experience
- Data Extraction, Curation, Privacy and Security Technologies
- Wholesalers
- Sales
- Sales/Consumption
- Reference data
- Trials registries
- Genomics
- Imaging
- Checklists
- Reference data
- Research experience
- Data Extraction, Curation, Privacy and Security Technologies
Data standardization and harmonization through OHDSI

What OHDSI is
- Open Source
- Community
- Data

Why Choose OHDSI/OMOP
- **Fast, reliable** studies across a series of datasets and data types
- **Reduced cost of ownership** including understanding coding schemes, writing statistical programs across databases or developing software
- **Expanded data access** via the OHDSI network and remote multi-center database studies

OHDSI Collaborators
- 3,758 collaborators
- >1,100 organizations
- 83 countries from 6 continents

OHDSI Network
- 534 data sources
- 49 countries
- 956M unique patient records

https://ohdsi.org/
Comparison of common data models

Balancing trade-offs in data management vs. analysis complexity

- **Common protocol**
- **Common structure**
- **Common conventions**
- **Common vocabularies**

### Complexity for data management
- **Jerboa** for 1 study
- **OHDSI**
- **pcornet**
- **i2b2**
- **HL7 FHIR**

### Complexity for analyst
- **Jerboa** for N studies
- **OHDSI**
- **pcornet**
- **i2b2**
- **HL7 FHIR**

- **Cohort identification**
- **Clinical characterization**
- **Population-level effect estimation**
- **Patient-level prediction**

- **Easier**
- **Harder**
Collaboration across standards

**OHDSI/FHIR**

**OMOP CDM**
- Dual source platform that supports both data science and application deployment
- Use of study results as actionable data to drive treatment decisions

**Improved Data Quality**
Leveraging the OMOP standard data models and data elements defined in FHIR ensures consistent and accurate data capture, which improves the validity and reliability of observational studies.

**Real-time Analysis**
Real-time analysis of FHIR-compliant data, which can be useful for real-world evidence generation and other applications.

**HL7 International and OHDSI Announce Collaboration to Provide Single Common Data Model for Sharing Information in Clinical Care and Observational Research**

Health Level Seven International (HL7) and the Observational Health Data Sciences and Informatics (OHDSI) network today announced a collaboration to address the sharing and tracking of data in the healthcare and research industries by creating a single common data model. The organizations will integrate HL7 Fast Healthcare Interoperability Resources (FHIR) and OHDSI’s Observational Medical Outcomes Partnership (OMOP) common data model to achieve this goal.

HL7 International CIO Dr. Charles Jaffe, M.D., Ph.D., underscored the significance of this partnering. “The Covid-19 pandemic has emphasized the need to share global health and research data,” he continued. “Collaboration with OHDSI is critical to solving this challenge and will help our mutual vision of a world in which everyone can securely access and use the right data when and where they need it.”

https://www.researchgate.net/figure/Data-Mapping-Concept-for-FHIR-to-OMOP-using-MEASUREMENT_fig4_354739998
Data Quality Dashboard (DQD)

**Description**

- Developed in 2019 by OHDSI
  - IQVIA part of core development team
- Follows the Kahn Framework
  - [https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5051581/](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5051581/)
- **3000+ checks** on plausibility, conformance, completeness
- Executed with each data refresh

**Deliverable**
FDA Best Federated Data Network Overview

**Study Investigations & Adverse Events**
- Studies:
  - Simple Rapid queries
  - Cohort characterization
  - Safety & Surveillance
  - Pharmacoepidemiology
  - Adverse Event Reporting

**Data Partners**
- Run studies
- Various data types (claims, EHR)

**Coordinating Center**
- Study protocol development
- Develop analytical packages
- External validation
- Coordinate data partner activities
- Program management

**Data Quality**
- Standardized data quality pipeline
- Establish data quality application for data quality assessment

**Scientific Advisory**
- Develop reproducible analytics tools and scientific methods
- Maintain data standardization and data quality standards
- Adoption of OMOP CDM globally

Premier, multi-center research collaborative driving large scale health analytics research
FDA BEST: COVID-19 Vaccine Safety Studies

Key outcomes and communication

Vascular outcomes (RCA)¹
- Four potential AESIs detected
- Adults 65 years and older
- Post-vaccination with Pfizer-BioNTech COVID-19 vaccines
- FDA safety communication – Jul 2021

Myocarditis/Pericarditis²
- Potential signal in young, male adults
- Post-vaccination with mRNA COVID-19 vaccines
- Study completion – Dec 2021

RCA in adolescents and adults aged 12-64 years³
- 17 outcomes monitored in 3 databases
- Myocarditis/pericarditis signaled in 2 of 3 databases
- Anaphylaxis signaled in all databases
- Study completion – Apr 2022

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2. Wong, Hui-Lee et al., Risk of myocarditis and pericarditis after the COVID-19 mRNA vaccination in the USA: a cohort study in claims databases. The Lancet, Volume 399, Issue 10342, 2191–2199
Key takeaways

Thank you for your attendance

01 Regulatory entities are becoming more interested in the use of real-world data not only for surveillance and safety, but for drug development and clinical research

02 OHDSI is an open-source community with data standardization and vocabulary harmonization

03 Standardized analytics allows for transparency and gaining of trust

04 OHDSI is a globally accepted methodology and continues to expand in the APAC region
Questions?