Trends in RWD/RWE and Data Standardization

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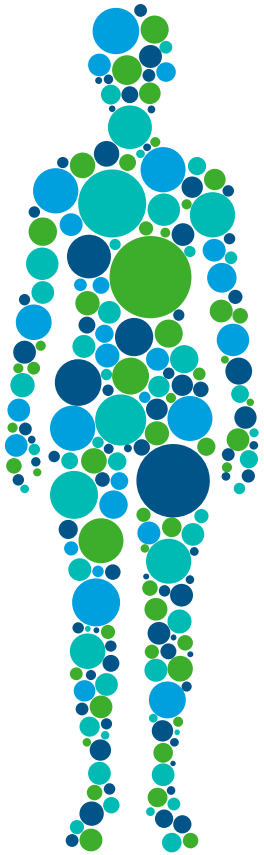
GM, Inteliquet

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

DARWIN EU Initiative
Started in 2022, pushing towards 2025 network startegy

Draft guidance 2021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

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

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

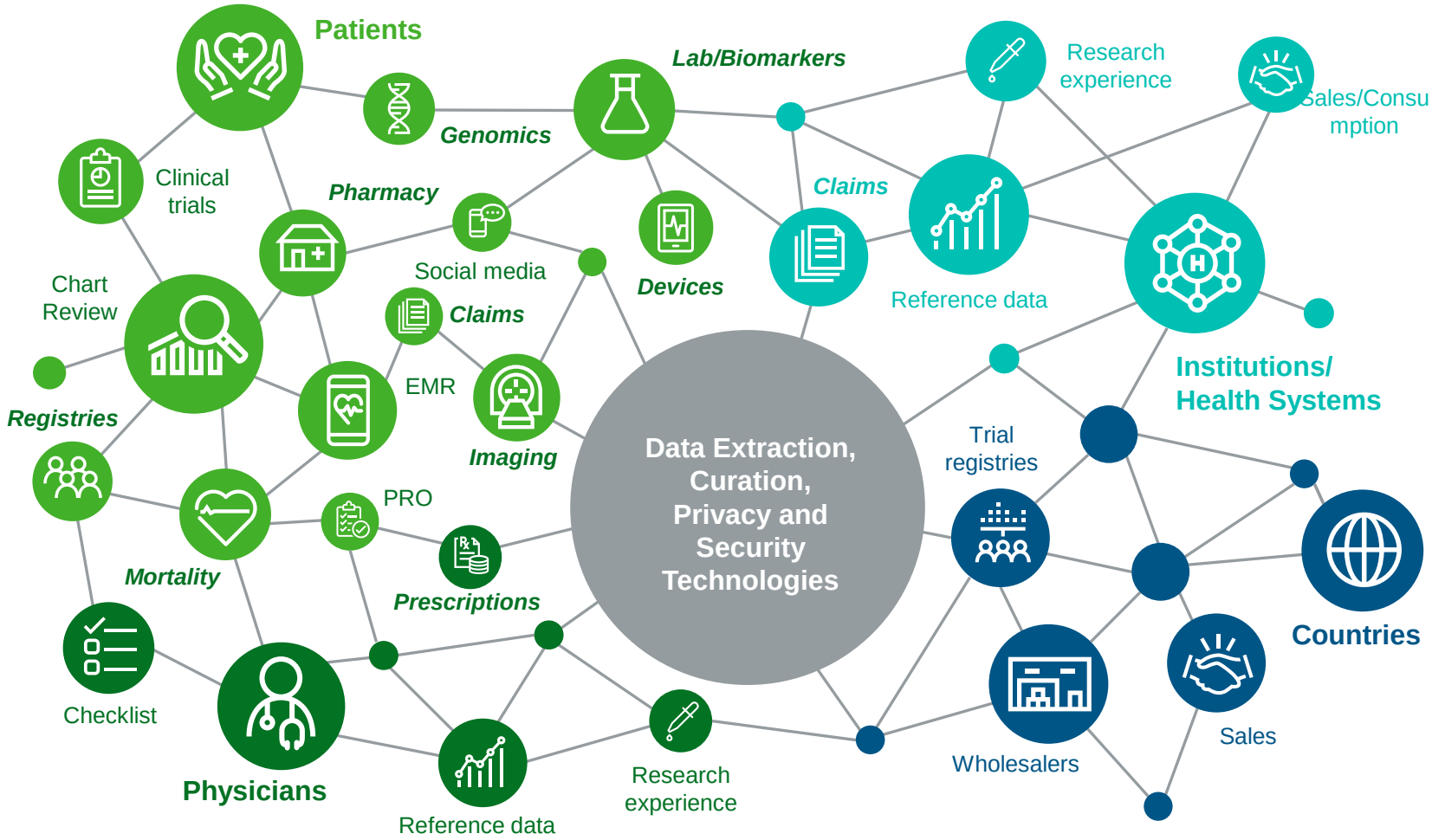
Registry Guidance
March 2021 – guidance on utilization of registry for application and reliablity

DISHA
Digital Information Security in Healthcare Act

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

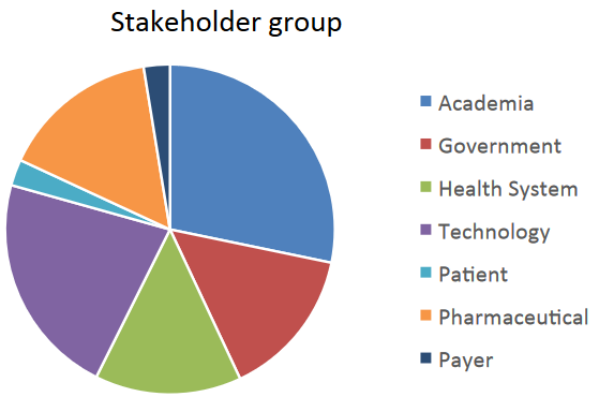
- Cohort Identification
- Clinical Characteristics
- Translational Research
- Prevalence & Incidence
- Drug Safety & Efficacy
- Comparative Effectiveness

Data standardization and harmonization through OHDSI

What OHDSI is

- Open Source
- Community
- Data

OHDSI
OBSERVATIONAL HEALTH DATA SCIENCES AND INFORMATICS



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

https://ohdsi.org/

OHDSI Collaborators

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

OHDSI Network

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

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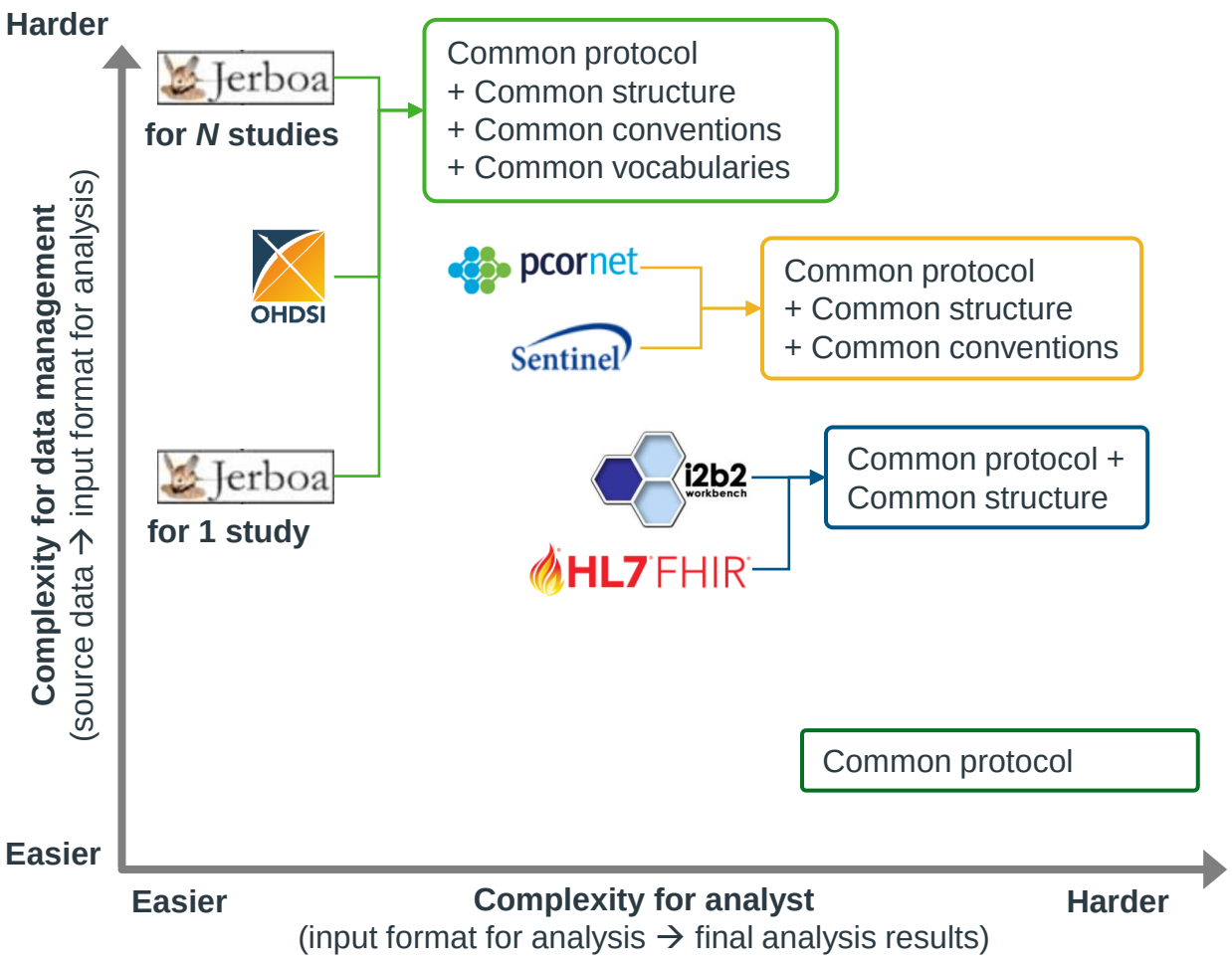
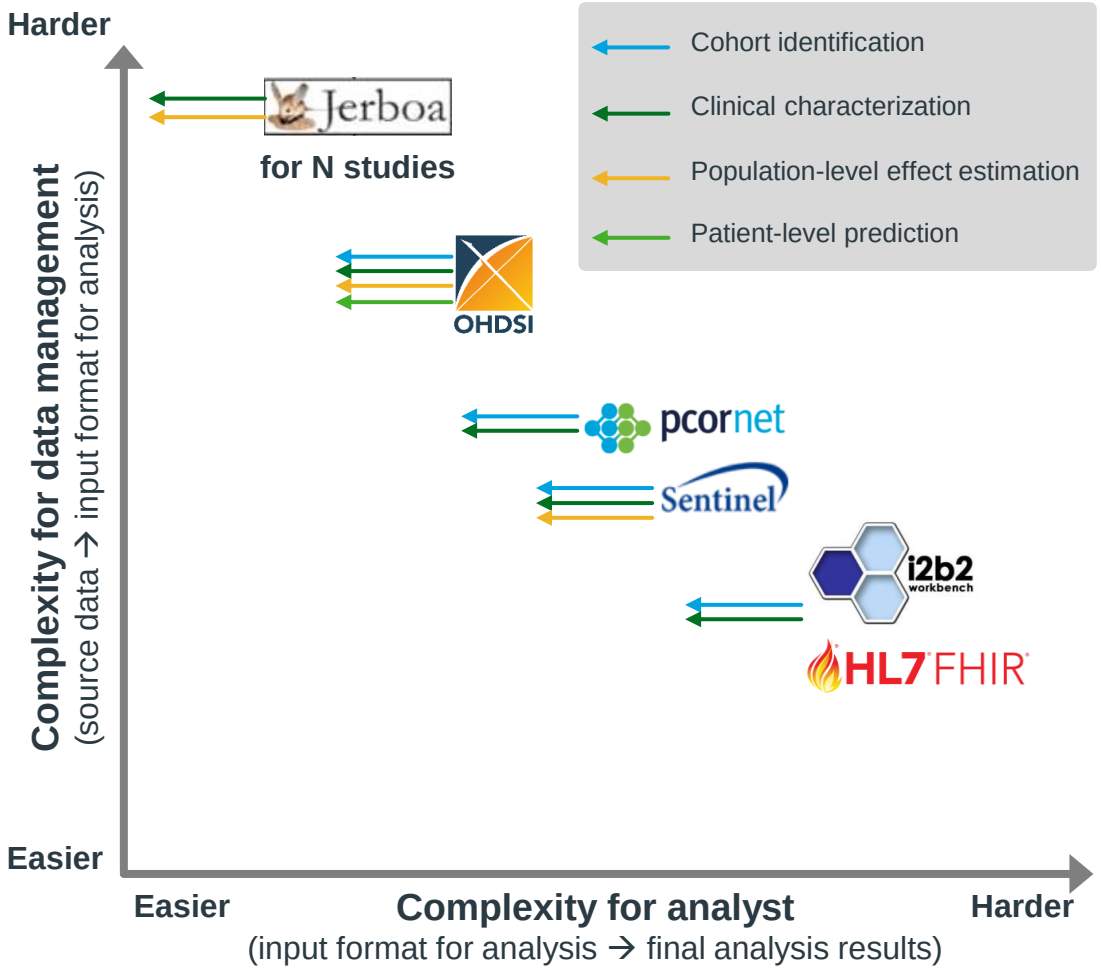
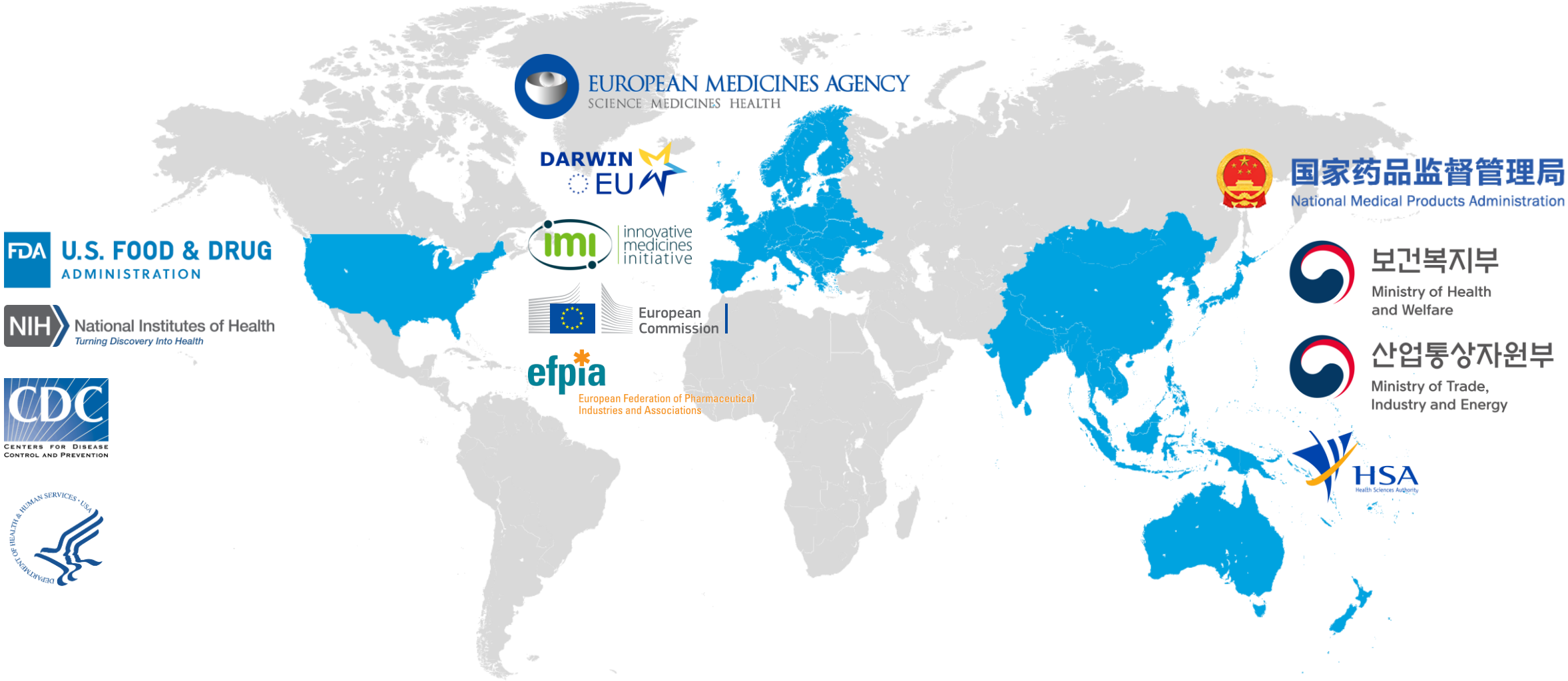
Comparison of common data models

Balancing trade-offs in data management vs. analysis complexity



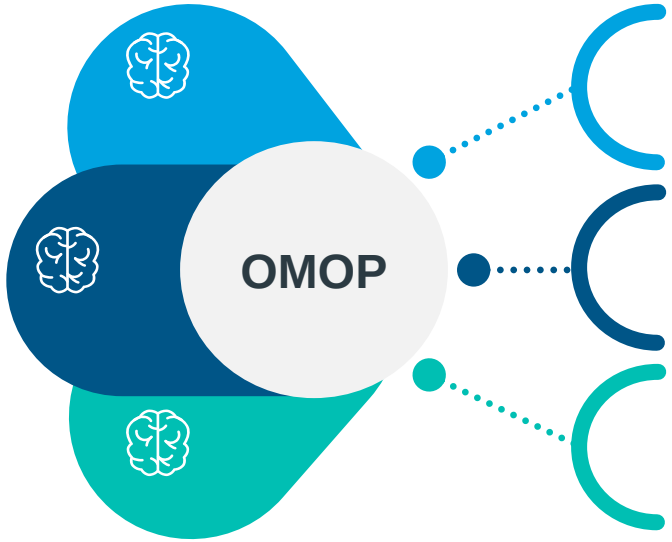


Global Government Adoption of OHDSI/OMOP



Collaboration across standards

OHDSI/FHIR



OMOP CDM

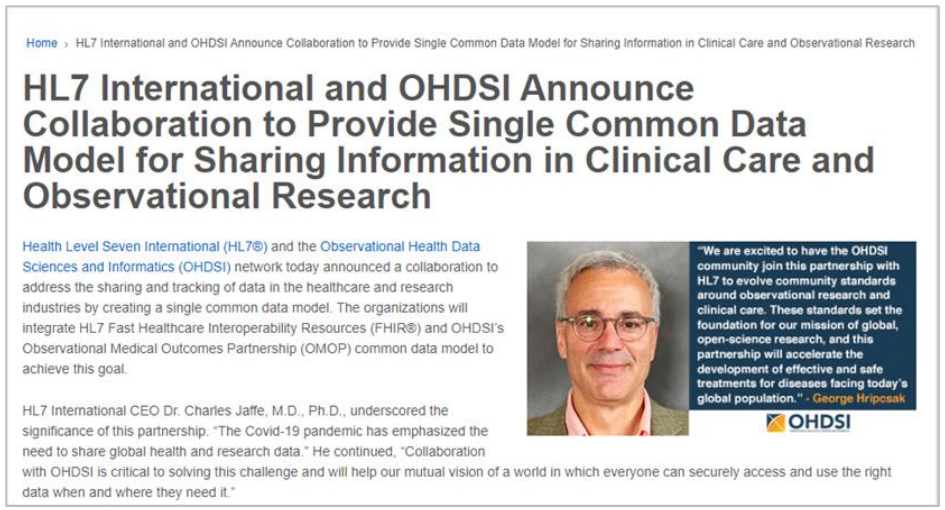
- Dual source platform that supports both data science and application deployment
- Use of study results as actionable data to drive treatment decisions

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.



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

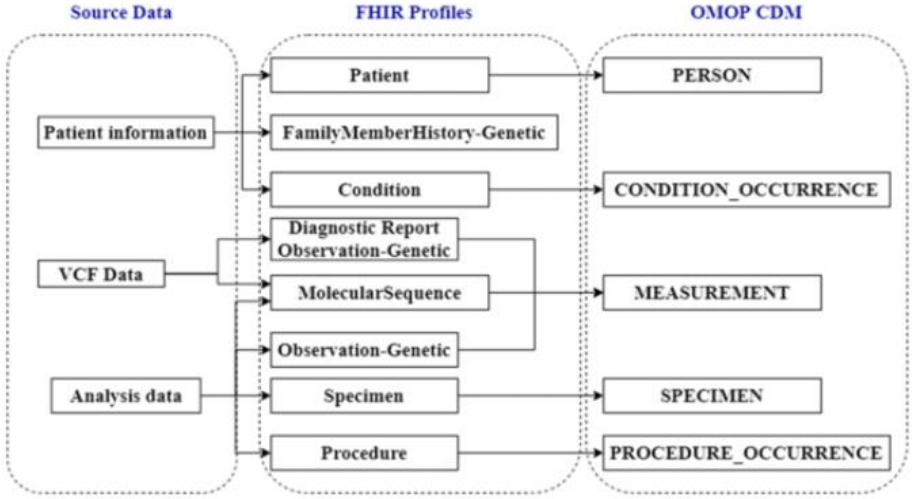
HL7 International and OHDSI Announce Collaboration to Provide Single Common Data Model for Sharing Information in Clinical Care and Observational Research

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.

"We are excited to have the OHDSI community join this partnership with HL7 to evolve community standards around observational research and clinical care. These standards set the foundation for our mission of global, open-science research, and this partnership will accelerate the development of effective and safe treatments for diseases facing today's global population." - George Hripcsak

OHDSI

HL7 International CEO Dr. Charles Jaffe, M.D., Ph.D., underscored the significance of this partnership. "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."



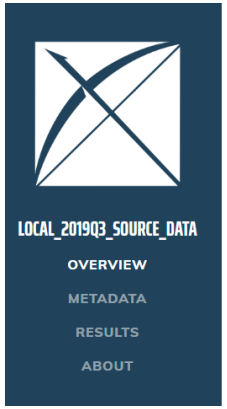
https://www.researchgate.net/figure/Data-Mapping-Concept-for-FHIR-to-OMOP-using-MEASUREMENT_fig4_354739998

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/
- ***3000+ checks*** on plausibility, conformance, completeness
- Executed with each data refresh

Deliverable



DATA QUALITY ASSESSMENT

LOCAL_2019Q3_SOURCE_DATA

Results generated at 2020-05-06 14:53:53 in 3 hours

	Verification				Validation				Total			
	Pass	Fail	Total	% Pass	Pass	Fail	Total	% Pass	Pass	Fail	Total	% Pass
Plausibility	1787	35	1822	98%	284	3	287	99%	2071	38	2109	98%
Conformance	516	15	531	97%	89	0	89	100%	605	15	620	98%
Completeness	287	20	307	93%	13	1	14	93%	300	21	321	93%
Total	2590	70	2660	97%	386	4	390	99%	2976	74	3050	**98%**

Column visibility | CSV

Show 5 entries

Search:

STATUS	CONTEXT	CATEGORY	SUBCATEGORY	LEVEL	DESCRIPTION	% RECORDS
FAIL	Verification	Completeness	None	FIELD	The number and percent of records with a value of 0 in the standard concept field unit_concept_id in the OBSERVATION table. (Threshold=5%).	100.00%
FAIL	Verification	Completeness	None	FIELD	The number and percent of records with a value of 0 in the source concept field dose_unit_concept_id in the DRUG_EXPOSURE table. (Threshold=10%).	100.00%
FAIL	Validation	Plausibility	Atemporal	CONCEPT	For a CONCEPT_ID 30969 (Testicular hyperfunction), the number and percent of records associated with patients with an implausible gender (correct gender = Male). (Threshold=5%).	98.26%
FAIL	Verification	Completeness	None	FIELD	The number and percent of records with a value of 0 in the standard concept field observation_concept_id in the OBSERVATION table. (Threshold=5%).	11.46%
FAIL	Validation	Plausibility	Atemporal	CONCEPT	For a CONCEPT_ID 200670 (Benign neoplasm of male genital organ), the number and percent of records associated with patients with an implausible gender (correct gender = Male). (Threshold=5%).	5.29%

Showing 1 to 5 of 74 entries (filtered from 3,050 total entries)

Previous 1 2 3 4 5 ... 15 Next

FDA Best Federated Data Network Overview

Study Investigations & Adverse Events

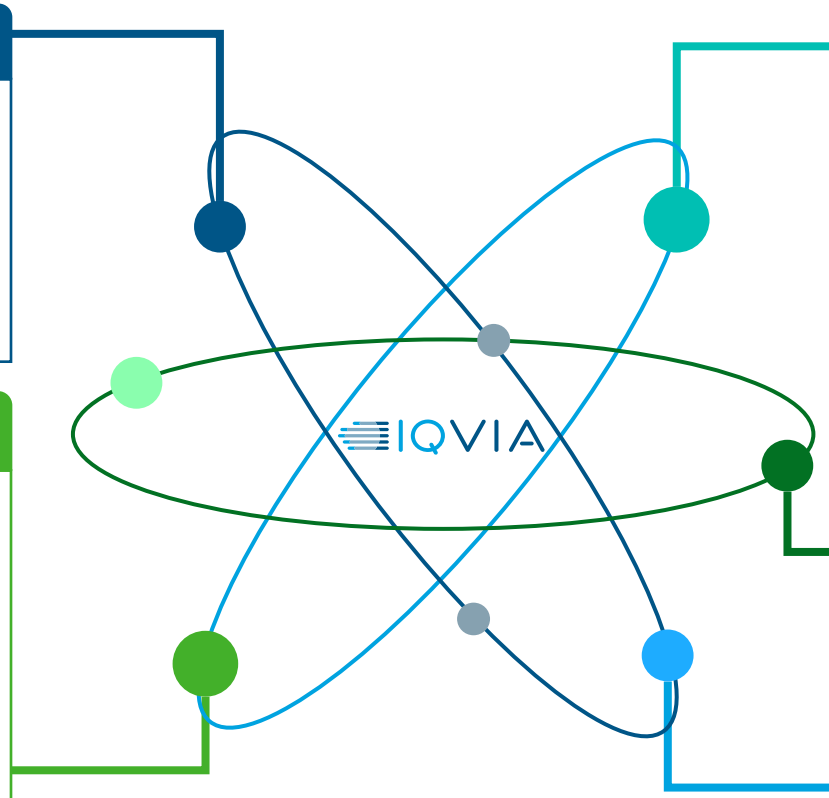
- Studies:
  - Simple Rapid queries
  - Cohort characterization
  - Safety & Surveillance
  - Pharmacoepidemiology
- Adverse Event Reporting

FDA U.S. FOOD & DRUG ADMINISTRATION

Data Partners

- Run studies
- Various data types (claims, EHR)

HealthCore · CVSHealth · PEDSnet · MARKET SCAN · Optum · MedStar Health · CMS.gov · Columbia University in the City of New York · OneFlorida+ Clinical Research Network · bhi Blue Health Intelligence · IBM



Coordinating Center

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

IQVIA

Data Quality

- Standardized data quality pipeline
- Establish data quality application for data quality assessment

IQVIA

Scientific Advisory

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

OHDSI

Premier, multi-center research collaborative driving large scale health analytics research

IQVIA

FDA BEST: COVID-19 Vaccine Safety Studies

Key outcomes and communication

Vascular outcomes (RCA)[1]

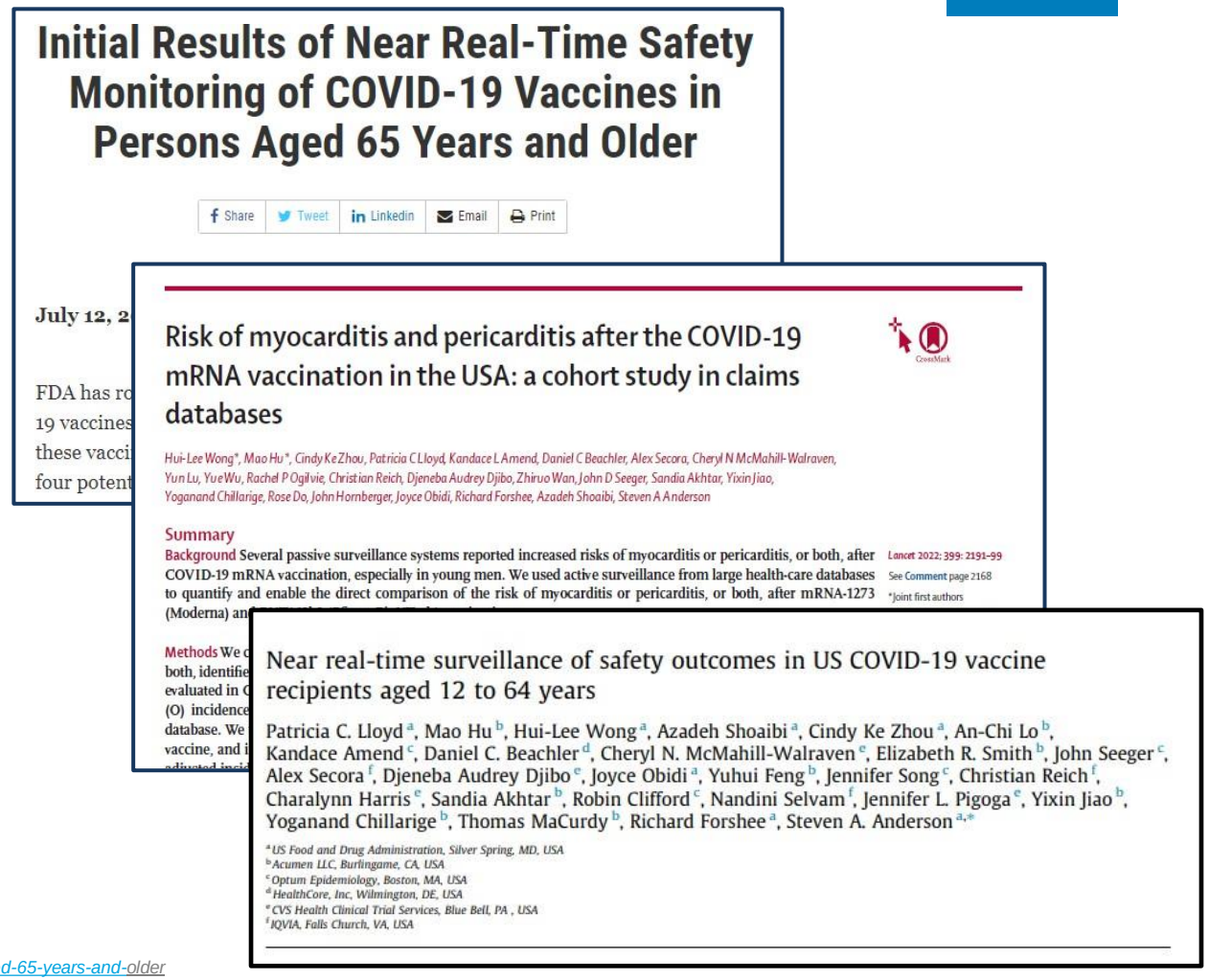
- Four potential AESIs detected
- Adults 65 years and older
- Post-vaccination with Pfizer-BioNTech COVID-19 vaccines
- FDA safety communication – **Jul 2021**

Myocarditis/Pericarditis[2]

- 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 years[3]

- 17 outcomes monitored in 3 databases
- Myocarditis/pericarditis signaled in 2 of 3 databases
- Anaphylaxis signaled in all databases
- Study completion – **Apr 2022**



Initial Results of Near Real-Time Safety Monitoring of COVID-19 Vaccines in Persons Aged 65 Years and Older

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July 12, 2

FDA has ro
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Risk of myocarditis and pericarditis after the COVID-19 mRNA vaccination in the USA: a cohort study in claims databases

Hui-Lee Wong*, Mao Hu*, Cindy Ke Zhou, Patricia C Lloyd, Kandace L Amend, Daniel C Beachler, Alex Secora, Cheryl N McMahill-Walraven, Yun Lu, Yue Wu, Rachel P Ogilvie, Christian Reich, Djeneba Audrey Djibo, Zhiruo Wan, John D Seeger, Sandia Akhtar, Yixin Jiao, Yoganand Chillarige, Rose Do, John Hornberger, Joyce Obidi, Richard Forshee, Azadeh Shoaibi, Steven A Anderson

Summary

Background Several passive surveillance systems reported increased risks of myocarditis or pericarditis, or both, after COVID-19 mRNA vaccination, especially in young men. We used active surveillance from large health-care databases to quantify and enable the direct comparison of the risk of myocarditis or pericarditis, or both, after mRNA-1273 (Moderna) an

Lancet 2022; 399: 2191–99
See Comment page 2168
*Joint first authors

Methods We
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Near real-time surveillance of safety outcomes in US COVID-19 vaccine recipients aged 12 to 64 years

Patricia C. Lloyd [a], Mao Hu [b], Hui-Lee Wong [a], Azadeh Shoaibi [a], Cindy Ke Zhou [a], An-Chi Lo [b], Kandace Amend [c], Daniel C. Beachler [d], Cheryl N. McMahill-Walraven [e], Elizabeth R. Smith [b], John Seeger [c], Alex Secora [f], Djeneba Audrey Djibo [c], Joyce Obidi [a], Yuhui Feng [b], Jennifer Song [c], Christian Reich [f], Charalynn Harris [e], Sandia Akhtar [b], Robin Clifford [c], Nandini Selvam [f], Jennifer L. Pigoga [e], Yixin Jiao [b], Yoganand Chillarige [b], Thomas MaCurdy [b], Richard Forshee [a], Steven A. Anderson [a,*]

[a] US Food and Drug Administration, Silver Spring, MD, USA
[b] Acumen LLC, Burlingame, CA, USA
[c] Optum Epidemiology, Boston, MA, USA
[d] HealthCore, Inc, Wilmington, DE, USA
[e] CVS Health Clinical Trial Services, Blue Bell, PA , USA
[f] IQVIA, Falls Church, VA, USA

1. https://www.fda.gov/vaccines-blood-biologics/safety-availability-biologics/initial-results-near-real-time-safety-monitoring-covid-19-vaccines-persons-aged-65-years-and-older
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
3. Lloyd PC, Hu M, Wong HL, Shoaibi A, Ke Zhou C, Lo AC, Amend K, Beachler DC, McMahill-Walraven CN, Smith ER, Seeger J, Secora A, Audrey Djibo D, Obidi J, Feng Y, Song J, Reich C, Harris C, Akhtar S, Clifford R, Selvam N, Pigoga JL, Jiao Y, Chillarige Y, MaCurdy T, Forshee R, Anderson SA. Near real-time surveillance of safety outcomes in US COVID-19 vaccine recipients aged 12 to 64 years. Vaccine. 2022 Sep 27:S0264-410X(22)01167-7. doi: 10.1016/j.vaccine.2022.09.060. Epub ahead of print. PMID: 36195472; PMCID: PMC9513329.

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?