IQVIA INSTITUTE FOR HUMAN DATA SCIENCE

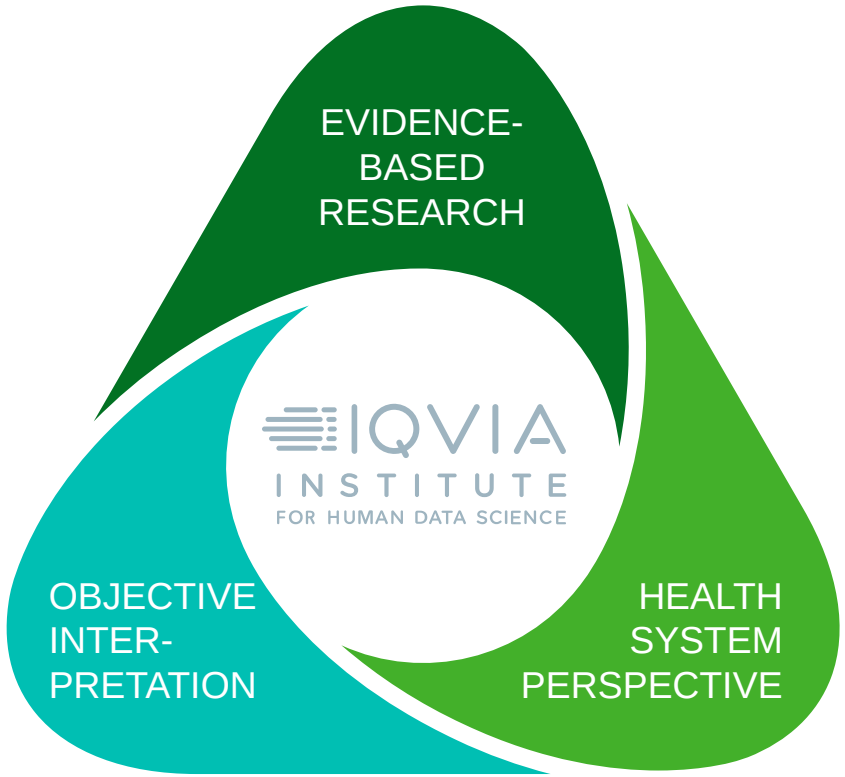
IQVIA Research Forum 2022

Pathways and Priorities for High Impact Health Research

October 2022

IQVIA Institute for Human Data Science contributes to advancing human health by generating rigorous, evidence-based research

- PROPRIETARY DATA SOURCES*
- SUBJECT MATTER EXPERTS
- ADVANCED ANALYTICAL SKILLS
- THIRD PARTY INFORMATION
- ACADEMIC PARTNERS
- EXTERNAL EXPERTS



- **LIBRARY** OF REPORTS
- **CONFERENCES** AND **FORUMS**
- PEER-REVIEWED **RESEARCH**
- **PRESS AND SOCIAL MEDIA** ENGAGEMENT

*No confidential sponsor or customer data is accessible or used

IQVIA INSTITUTE

Support for academic researchers

How researchers can benefit from IQVIA data

Through collaboration with the IQVIA Institute, researchers have access to a broad range of proprietary databases and tools to support independent research, discovery work, and requirement development for future funded studies.

Collaborating with the IQVIA Institute

Researchers interested in collaborating with the IQVIA Institute on specific research studies should contact us at **info@iqviainstitute.org.**

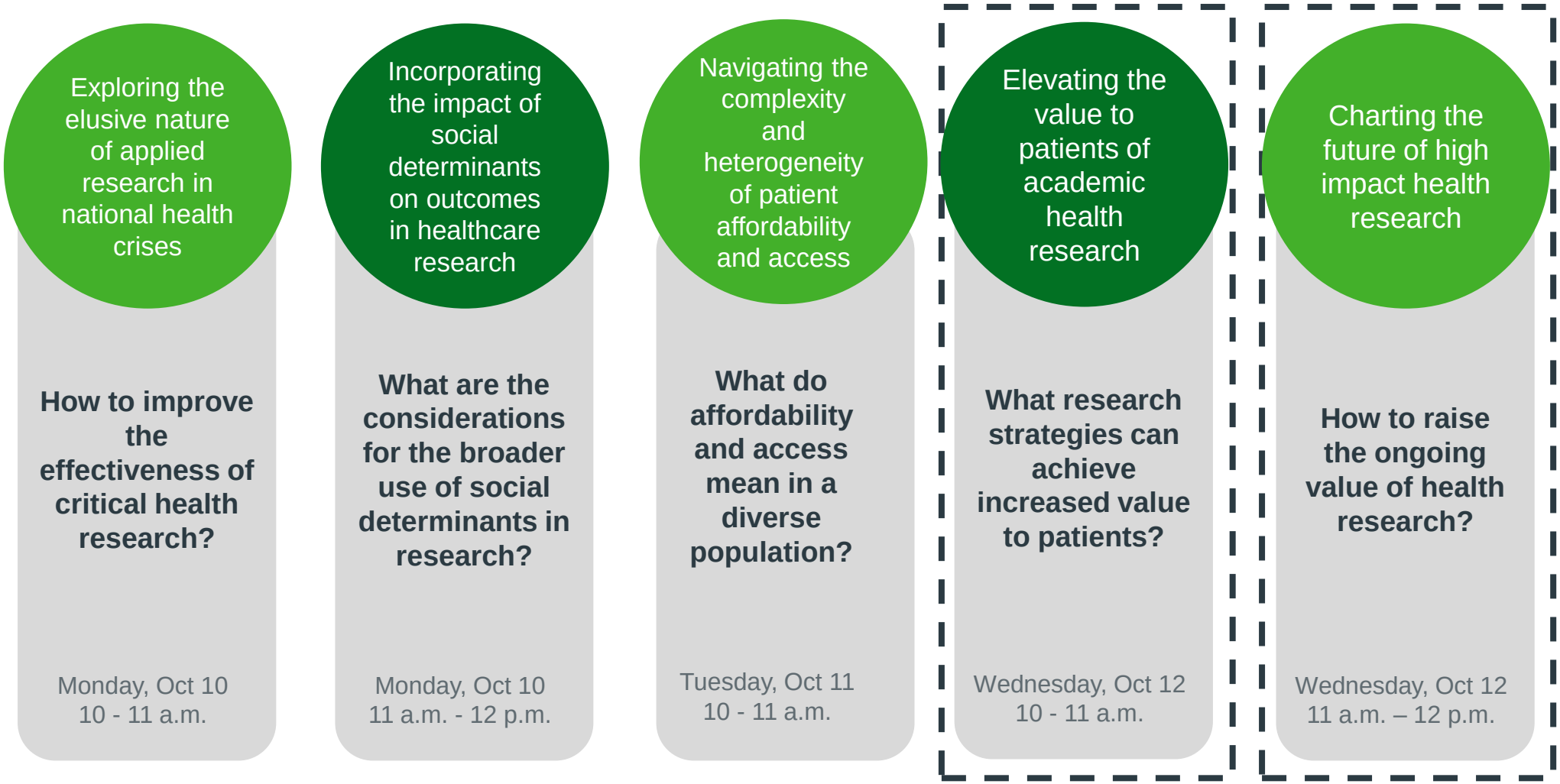
Detailed information is available on our web site at **iqviainstitute.org** under Research Support.

IQVIA data assets frequently used in academic research

1. **Formulary Impact Analyzer:** Pharmacy claims with insight into paid, rejected or reversed adjudication status.
2. **Longitudinal Prescription Claims (LRx):** Prescription claims from retail, mail and long-term care pharmacies.
3. **Medical and Institutional Claims (Dx and Hx):** Unadjudicated office and institutional medical claims.
4. **MIDAS:** Global pharmaceutical sales at a country and therapeutic level.
5. **National Prescription Audit (NPA):** Nationally projected prescription volume from retail, mail and long-term care pharmacies.
6. **National Sales Perspectives (NSP):** Nationally projected ship-to transaction volume and revenue to all retail and non-retail entities.
7. **OneKey:** Comprehensive healthcare organizational and professional affiliation data.
8. **Pharmetrics Plus for Academics:** Longitudinal health plan data for adjudicated claims.

Pathways and Priorities for High Impact Health Research

IQVIA Research Forum 2022



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Elevating the value to patients of academic health research

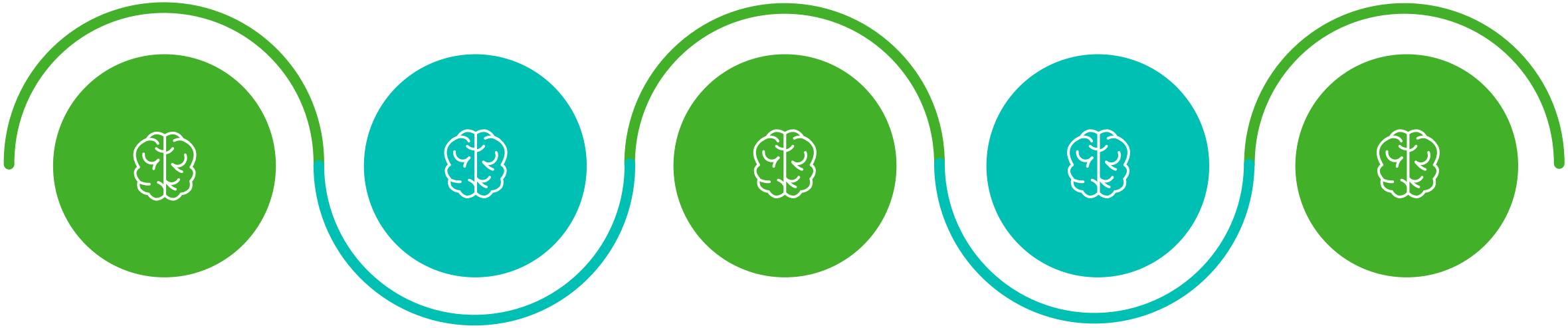
Panelists

Christian Reich, MD
Professor of the Practice, Visiting Scientist, OHDSI
The Roux Institute, Northeastern University

Joseph (Joe) Ross, MD, MHS
Co-founder
MedRxiv

Moderator: Murray Aitken, Executive Director, IQVIA Institute

How Do We Determine Whether Academic Research Brings Value to Patients?



Research design

Is the research meaningful to patients? Have patients been involved in the design of the research project?

Endpoints

Are the defined clinical endpoints of the study relevant to patients? Have the relevant patients been consulted for their perspective and experience? Which stakeholders have interest in which endpoints?

Outcomes

Are the outcomes of the study meaningful and relevant to patients? Is the focus of the research on process, organization or patient outcomes?

Decision-making

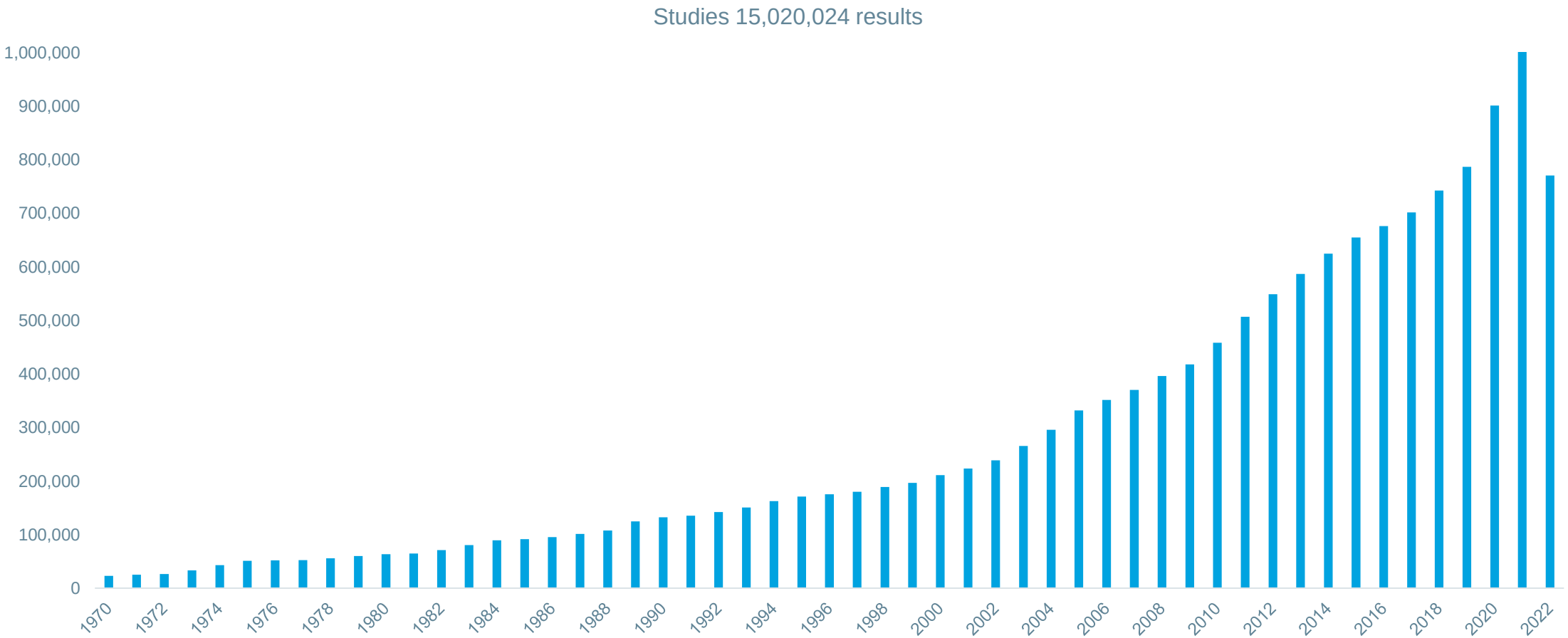
Which shared decisions between patient and physician are likely to be considered based on the research results?

Actions

Which actionable clinical steps does the research result point to?

Volume of Health Research Growing Rapidly Year over Year

PubMed Annual Rate of Published "Studies" 1970 - 2022



Source: PubMed, October 3, 2022

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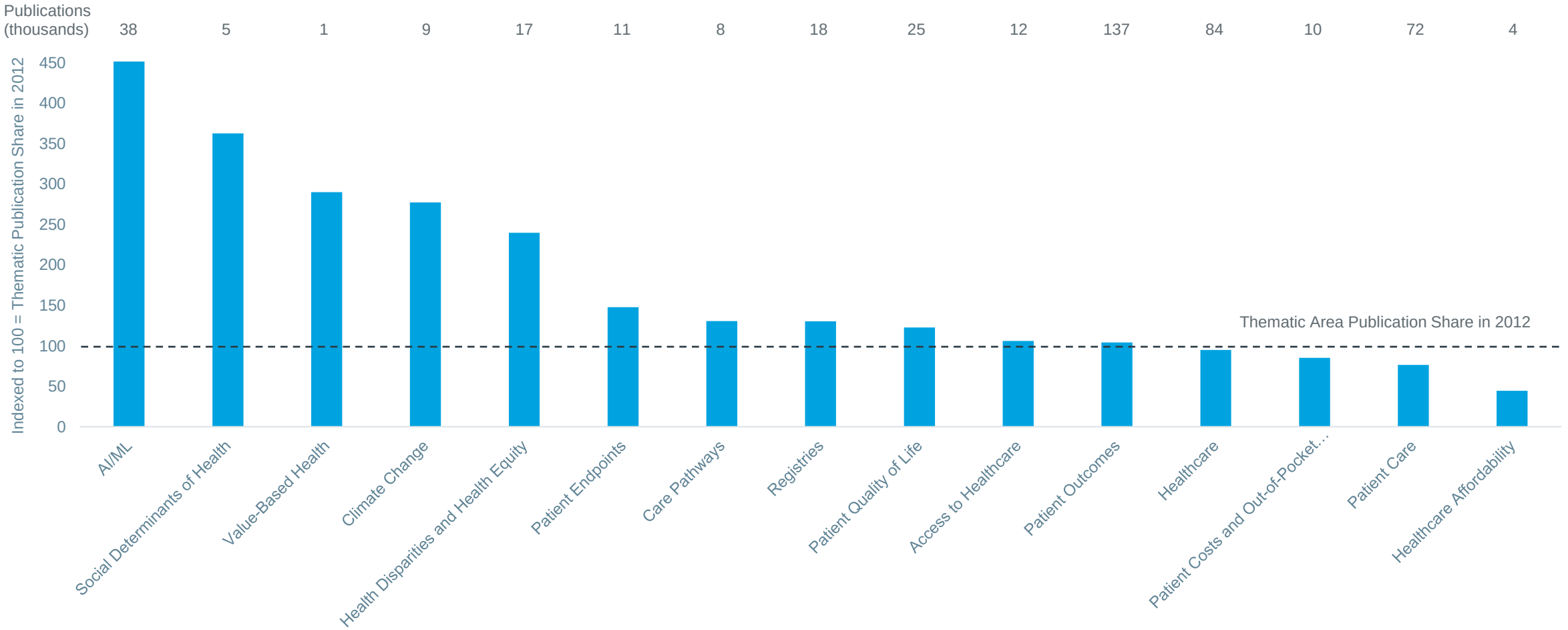
Higher Growth Areas of Research are Reflecting Current Issues

Publications in 2022 Indexed to 2012 Total Publication Share by Thematic Area



Source: PubMed, October 6, 2022

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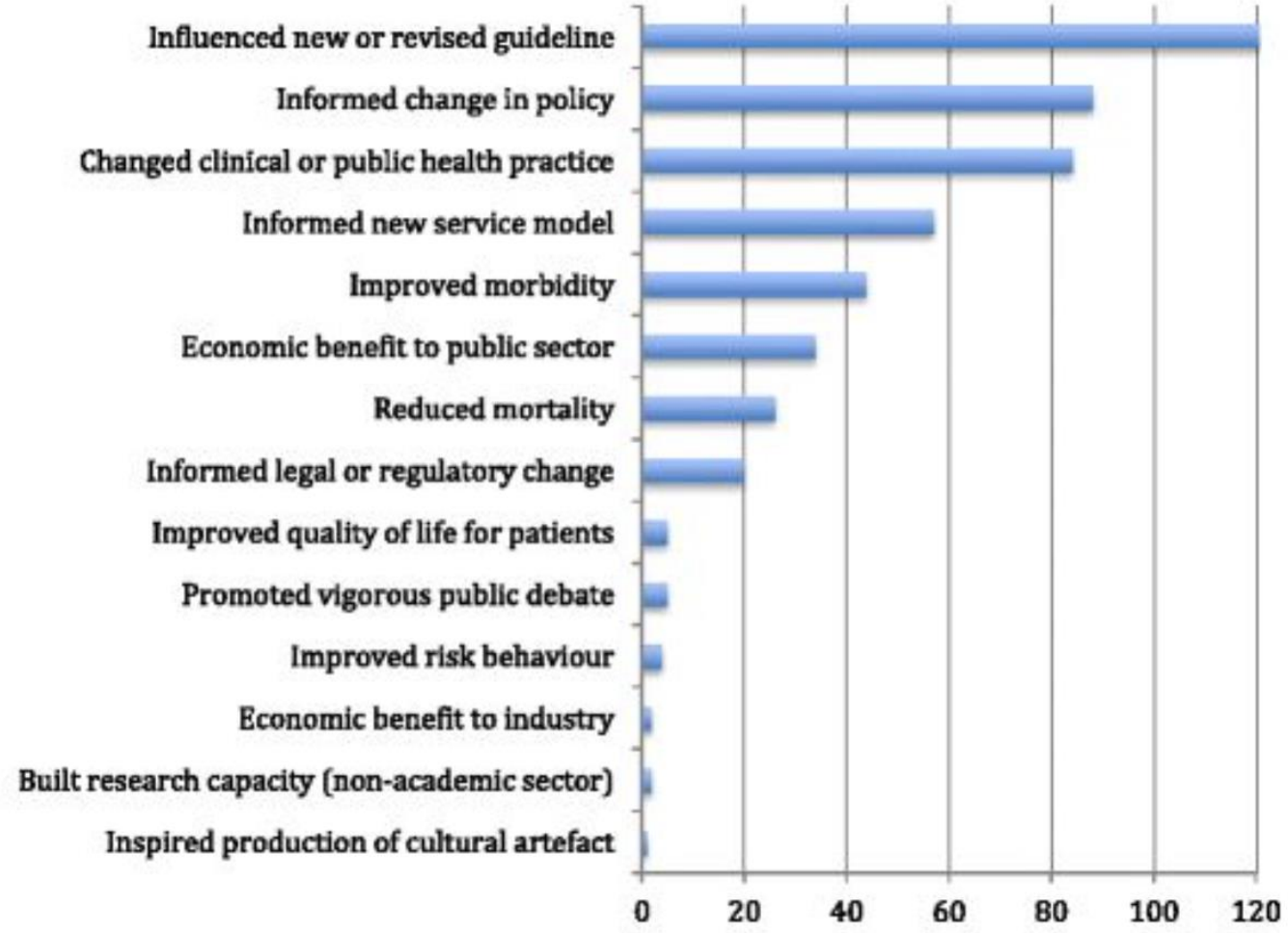
Medical Research Tends to Focus More on Processes than Outcomes



An analysis of the impacts described in 162 case studies submitted from community-based health sciences as part of the 2014 Research Excellence Framework. The majority described changes in processes rather than outcomes

Source: Richard Smith: Measuring research impact – all the rage but hard to get right. July 30.2018. British Medical Journal. https://blogs.bmj.com/bmj/2018/07/30/richard-smith-measuring-research-impact-rage-hard-get-right/

Patient Perspectives on Gaps in Health Research

"Everybody claims to have patient-relevant endpoints, but they are not always based on science and they are not always relevant to patients."

Bettina Ryll, Chair of Melanoma Patient Network Europe
(At IQVIA Institute, Pre-ESMO Symposium, September 9, 2022)

The C.D.C. found that record numbers of children under 5 had been hospitalized during the Omicron surge, underscoring the need for vaccines. But it also said that 90 percent of Americans could safely stop wearing masks in public indoor spaces, even in schools with young children.

Confusing, right?

NY Times, Virus Briefing,
March 10, 2022

"By me being a minority, a Black male, I know there are different medications that will help me more than say an Asian or Caucasian, and I ask the doctor… 'Have there been any studies done on this particular blood pressure medication that's geared towards minorities?' and he said 'Yes.' I said 'Well, which one do you think will work best for me?' "

Quote from patient focus group study. The American Journal of Bioethics.⁎

"Yeah, I suppose that's the key thing is making sure that patients have the chance to give their views, and that those views are listened to.... kind of more practical things. ... the kind of outcomes that are relevant in their life, you know, the idea of looking beyond just the clinical outcomes".

Quote from: "The patient's view on rare disease trial design – a qualitative study." ⁎ ⁎

Source: ⁎ Kelley M et al. Patient Perspectives on the Learning Health System: the Importance of Trust and Shared Decision Making. The American Journal of Bioethics, 25 August 2015. https://www.tandfonline.com/doi/full/10.1080/15265161.2015.1062163
⁎ ⁎ Gaasterland, CMV et al. The Patients's View on Rare Disease Trial Design – A Qualitative Study." Orphanet Journal of Rare Diseases. 7 February 2019, https://ojrd.biomedcentral.com/articles/10.1186/s13023-019-1002-z

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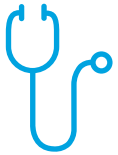
Potential Sources of “Leakage” in Health Research Patient Value

Where are the gaps in the evidence chain?

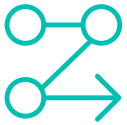
Research design

Research is not always designed to be meaningful to patients by measuring impact for or value to patients.



Capitalize on available data

Research often does not capitalize on available data, for example data from large databases and patient registries.



Novel research methods

Novel research methods that can elucidate value to patients in the real world as opposed to a clinical trials setting, for example real-world evidence, is not always applied in health research.

Enabling technologies

Novel enabling technologies that can demonstrate potential value in the real world, such as AI and ML, are complex and require new skills of researchers.

Medical journal process

The medical journal publication process is slow, cumbersome and rather antiquated.

Patient expertise

Medical journal editors don’t always engage patient-experts in the review of studies.

New Pathway for Elevating Value of Research to Patients

Employ patient-reviewers

Employing experts in patient and real-world issues in the editorial review process

Co-design research with people affected

Bringing in people who are affected by the issues and interventions that are the subject of health research to help co-design the research

Develop measurement guidelines

Developing consensus guidelines for measuring impact and value of research to patients, patient populations and health systems

Engage underrepresented populations

Engaging underrepresented populations and communities hitherto have been overlooked in health research

Create review frameworks

Creating and implementing new frameworks for assessing impact of science

OHDSI Overview

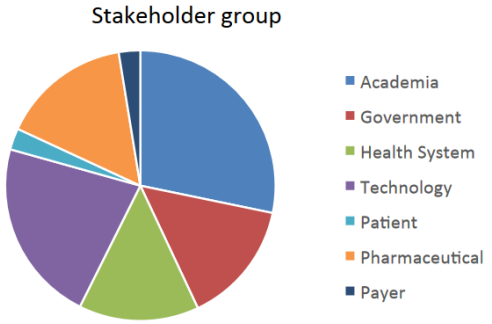
Christian Reich

OHDSI

OBSERVATIONAL HEALTH DATA SCIENCES AND INFORMATICS

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:

- 2,900 users
- 29 workgroups
- 46,900 posts on 5,700 topics

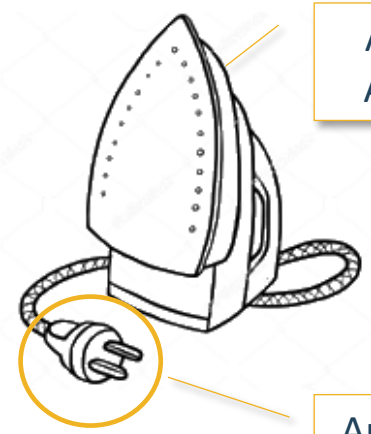
OHDSI Network:

- >320+ databases
- 34 countries
- >2.7B patient records, >369M ex-US

How OMOP Works

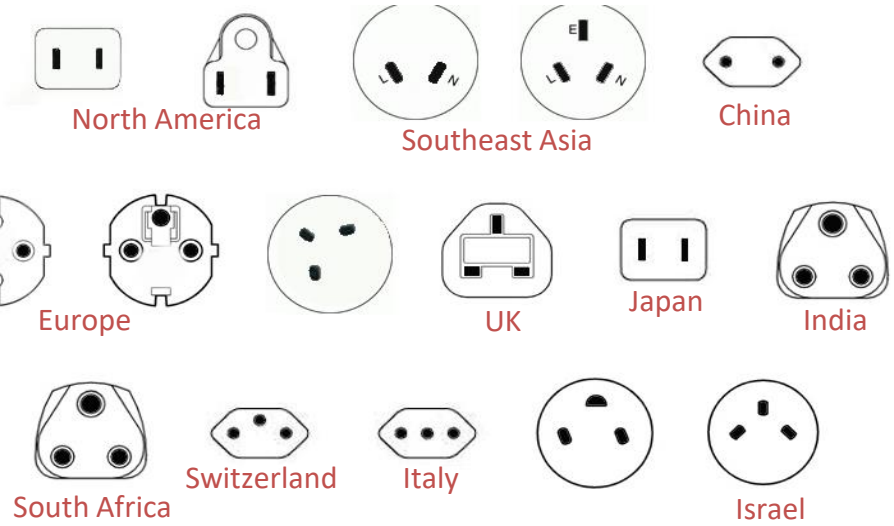
Current Approach: “One Study – One Script“

"What's the adherence to my drug in the data assets I own?"

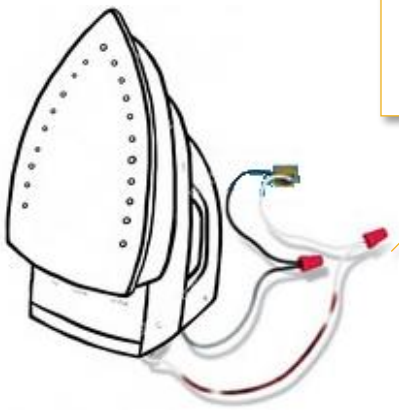


Analytical method: Adherence to Drug

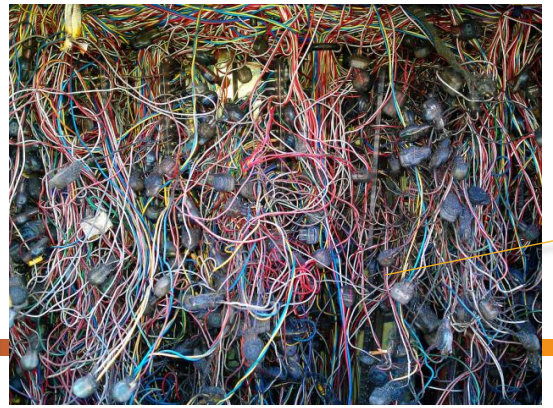
Application to data



Current solution:

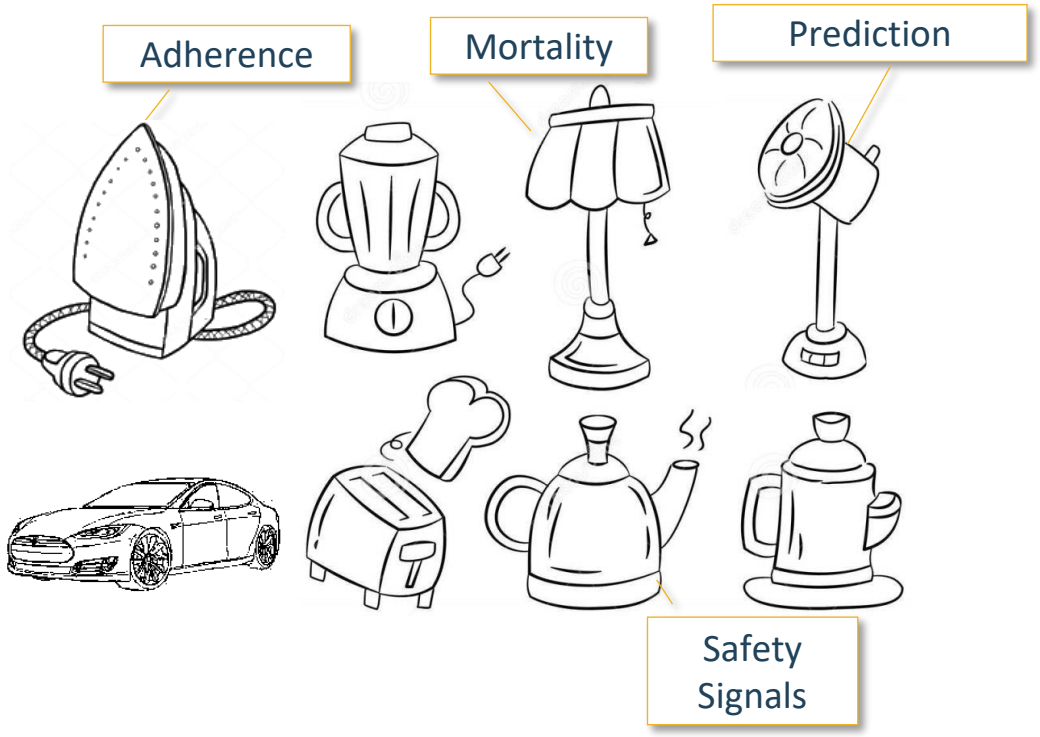


One SAS or R script for each study

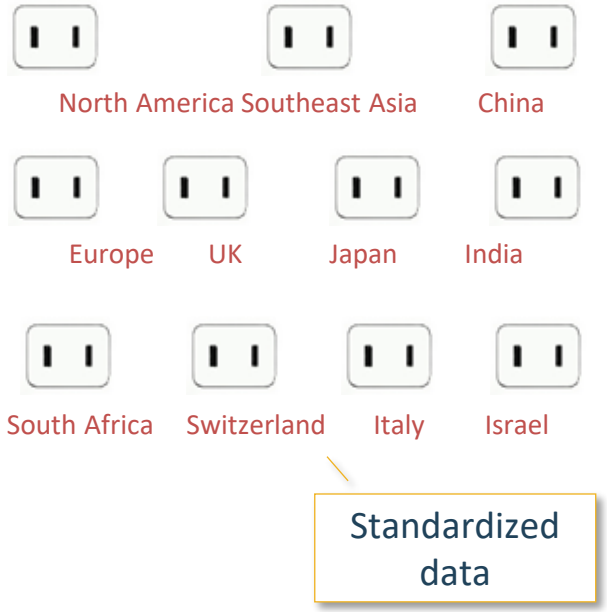


- Not scalable
- Not transparent
- Expensive
- Slow
- Prohibitive to non-expert routine use

The OHDSI Approach

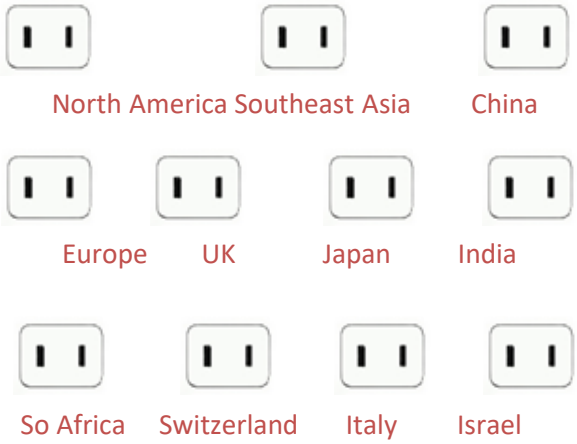


OHDSI Tools



OMOP CDM

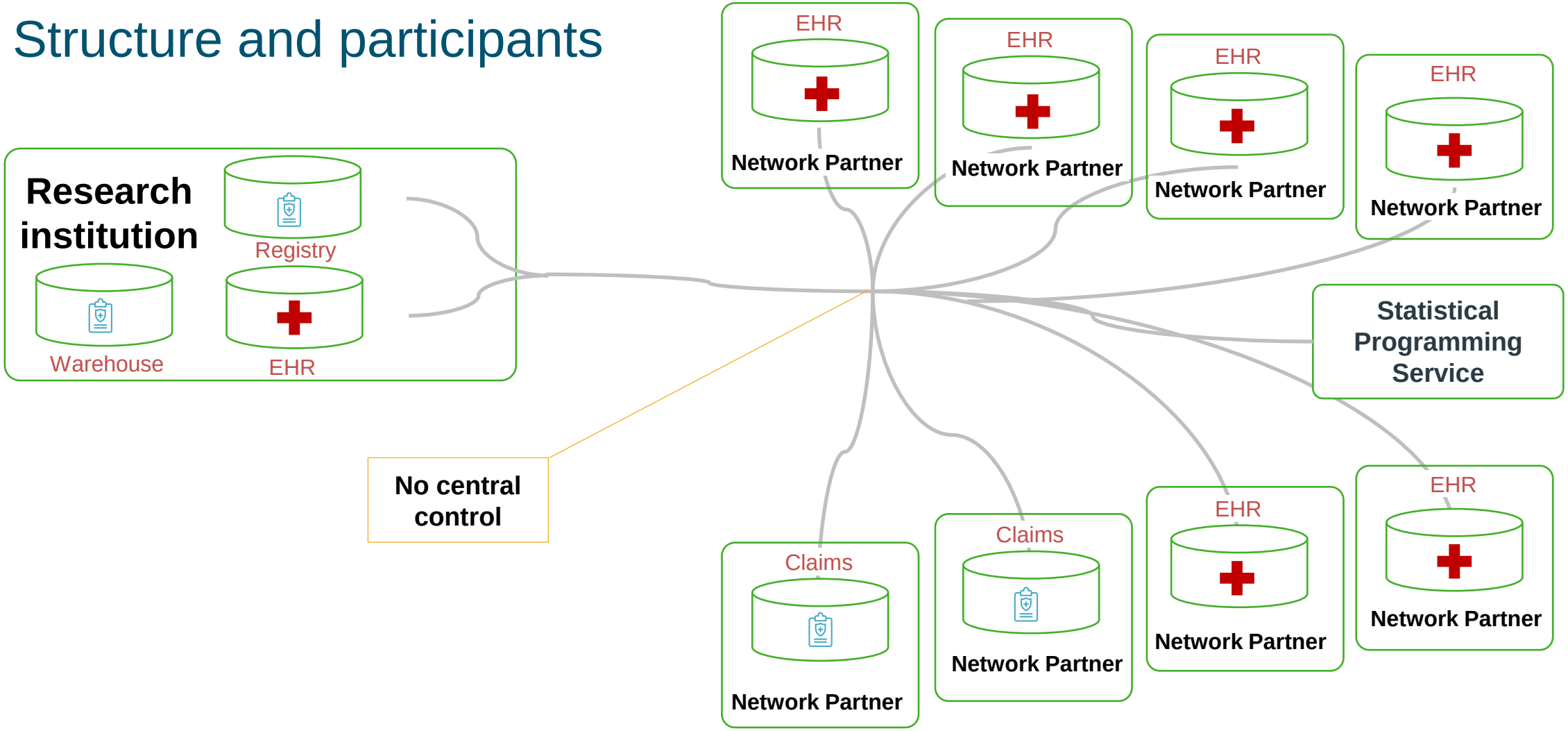
Analytics can be remote



Analytics can be behind firewall

OHDSI Research Network

Structure and participants




OHDSI
OBSERVATIONAL HEALTH DATA SCIENCES AND INFORMATICS

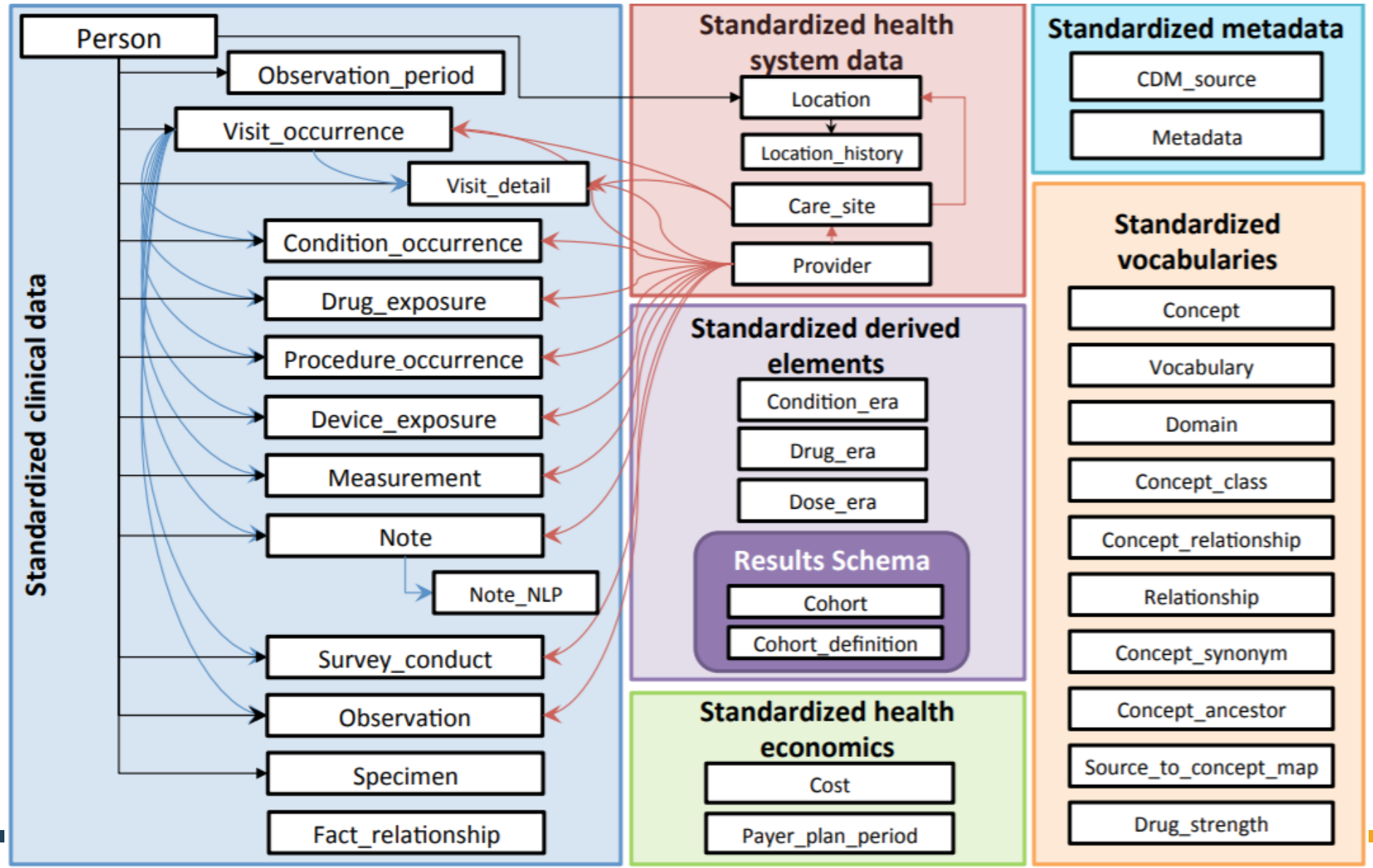
OHDSI Tools

Opportunities for Standardization

Protocol

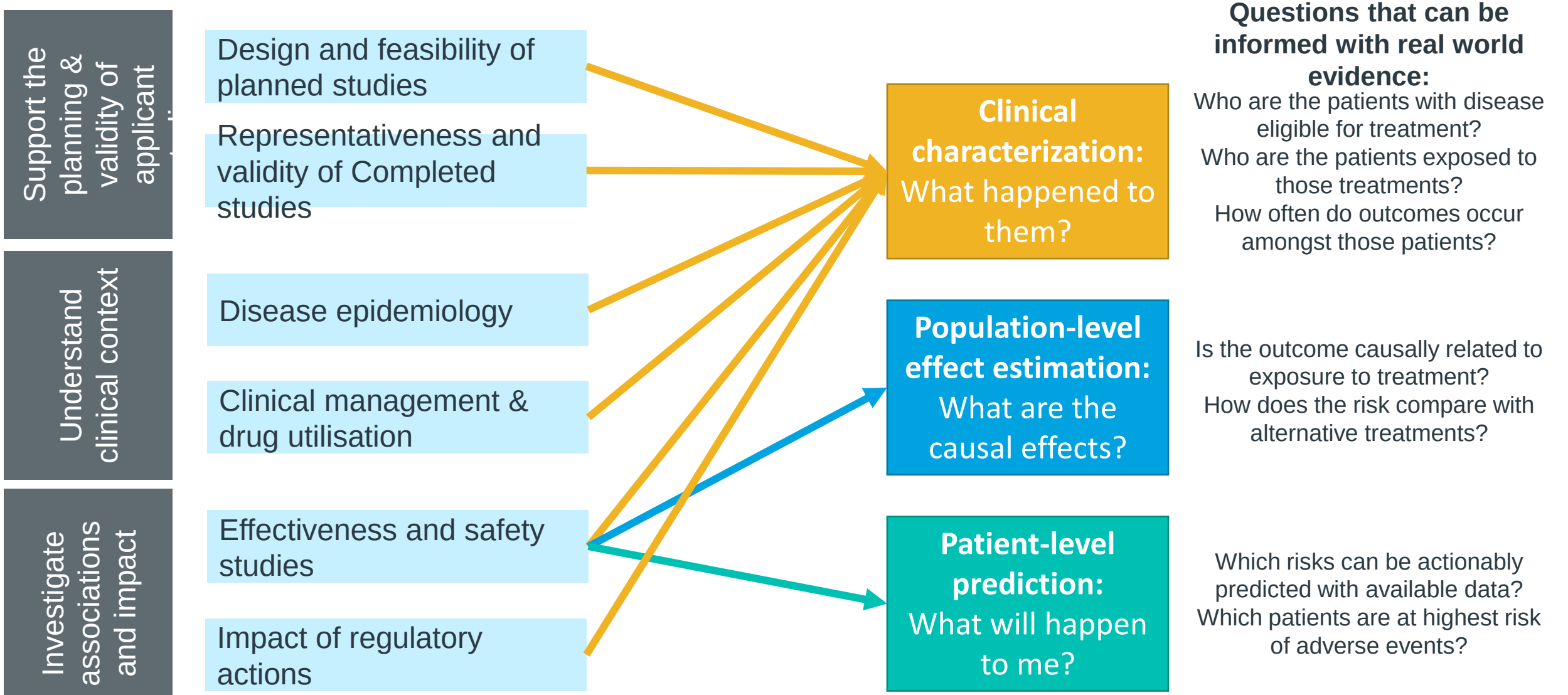
Data structure	tables, fields, data types
Data content	vocabulary to codify clinical domains
Data semantics	conventions about meaning
Cohort definition	algorithms for identifying the set of patients who meet a collection of criteria for a given interval of time
Covariate construction	logic to define variables available for use in statistical analysis
Analysis	collection of decisions and procedures required to produce aggregate summary statistics from patient-level data
Results reporting	series of aggregate summary statistics presented in tabular and graphical form

Standard Content: OMOP Common Data Model



Applications

Mapping regulatory use cases to evidence types



Expanding the proactive use of real-world evidence for study planning and validity

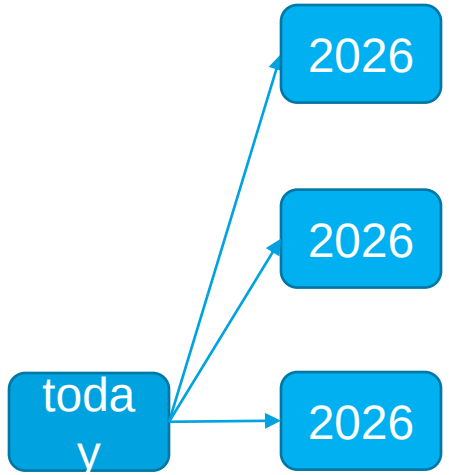
~seconds — Anticipatory

~minutes — Prepared

~hours — Responsive

~days — Enabled

~weeks, months, years — Reactive Bespoke



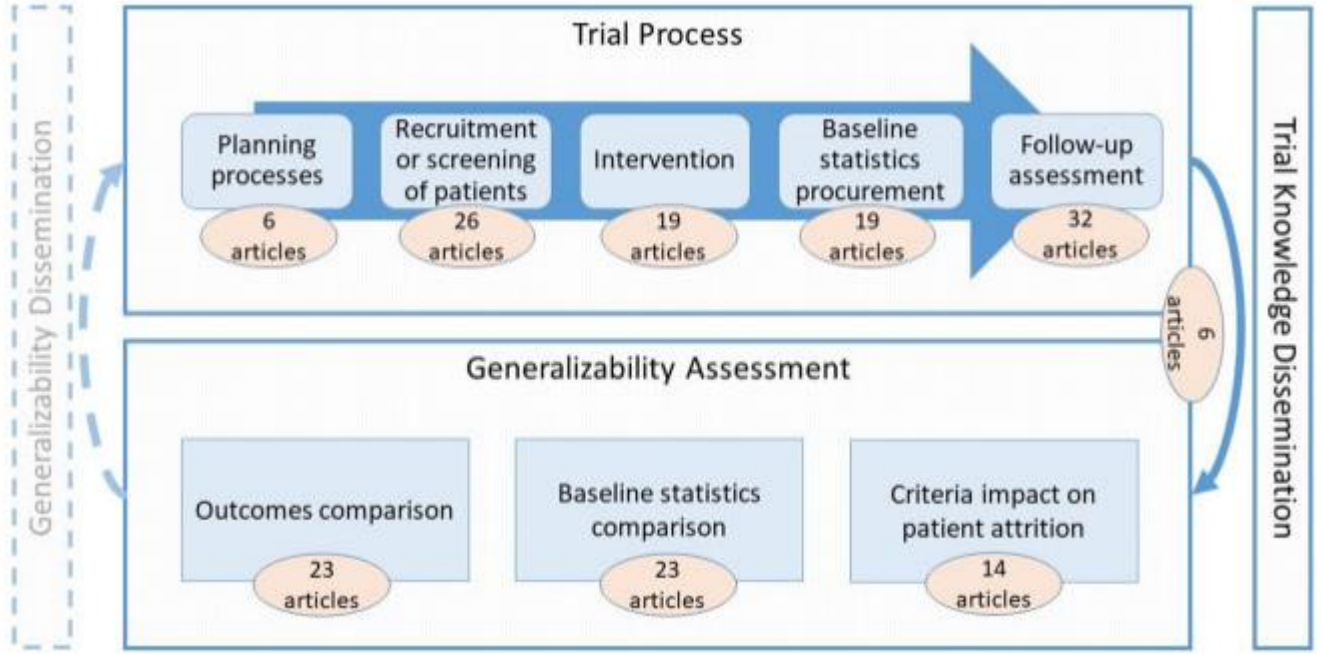
Support the planning & validity of applicant studies

Journal of the American Medical Informatics Association, 28(1), 2021, 144–154
doi: 10.1093/jamia/ocaa224
Advance Access Publication Date: 4 November 2020
Review

Review

Contemporary use of real-world data for clinical trial conduct in the United States: a scoping review

James R. Rogers,[1] Junghwan Lee,[1] Ziheng Zhou,[2] Ying Kuen Cheung,[3] George Hripcsak,[1,4] and Chunhua Weng[1]



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today → 2026, 2026, 2026

Support the planning & validity of applicant studies

Journal of Biomedical Informatics 119 (2021) 103822

Contents lists available at ScienceDirect

Journal of Biomedical Informatics

journal homepage: www.elsevier.com/locate/yjbin

Original Research

Clinical comparison between trial participants and potentially eligible patients using electronic health record data: A generalizability assessment method

James R. Rogers [a], George Hripcsak [a,b], Ying Kuen Cheung [c], Chunhua Weng [a,*]

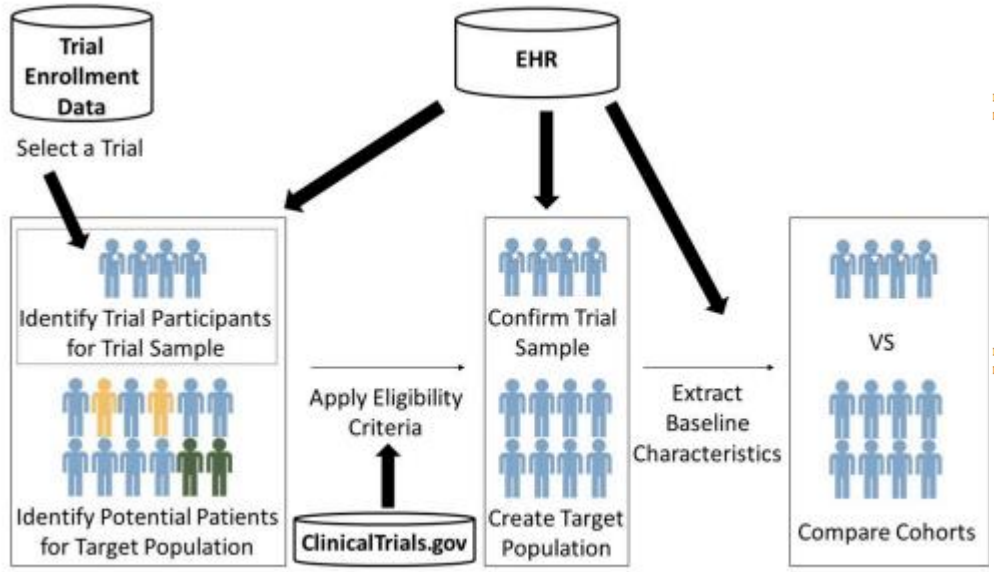

Fig. 1. Overview of study methodology.

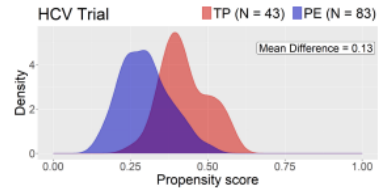

Fig. 3. Distribution of propensity scores between trial participants (TP) and potentially eligible (PE) patients for the hepatitis C virus (HCV) trial.

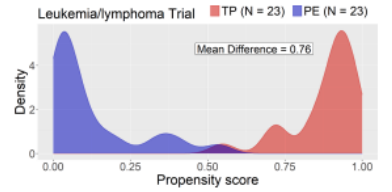

Fig. 4. Distribution of propensity scores between trial participants (TP) and potentially eligible (PE) patients for the leukemia/lymphoma trial.

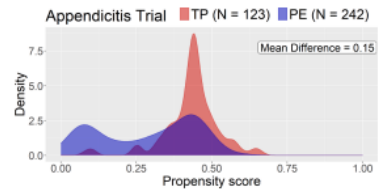

Fig. 5. Distribution of propensity scores between trial participants (TP) and potentially eligible (PE) patients for the appendicitis trial.

Expanding the proactive use of real-world evidence for understanding clinical context

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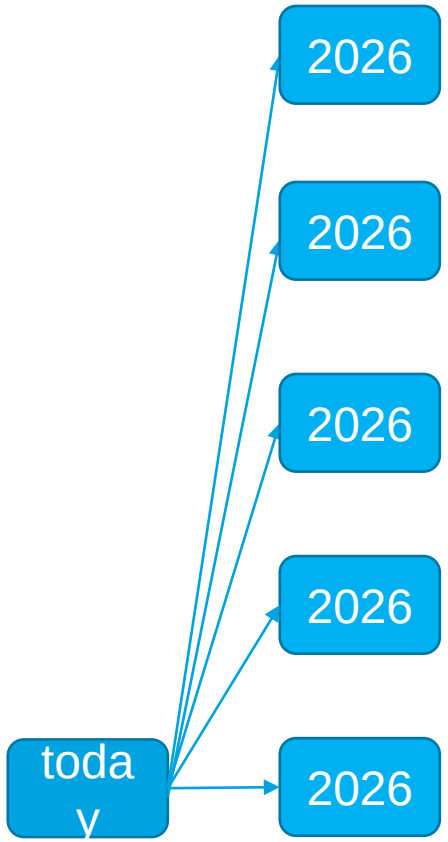
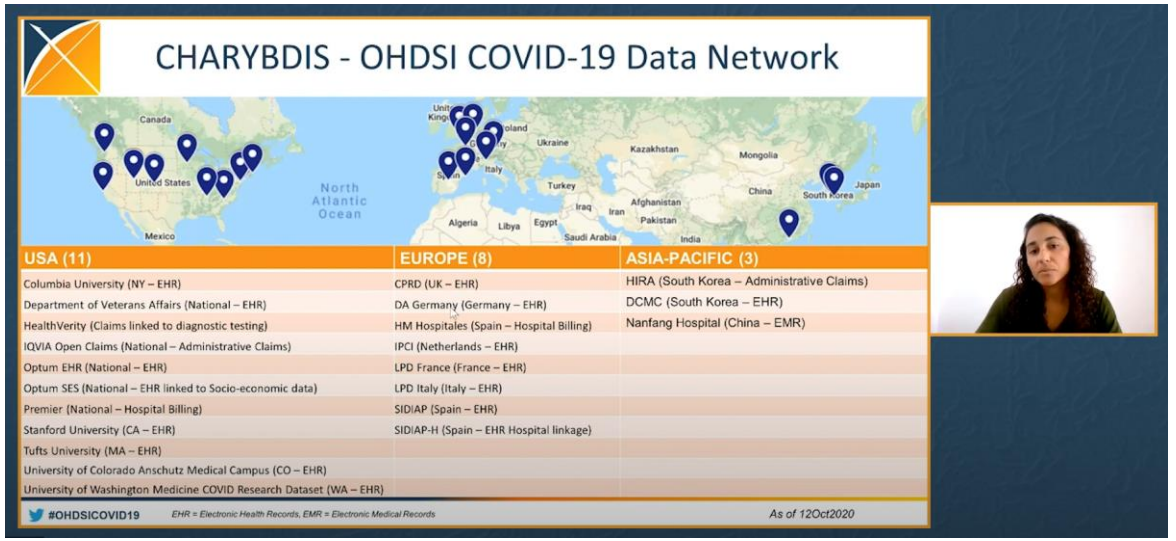
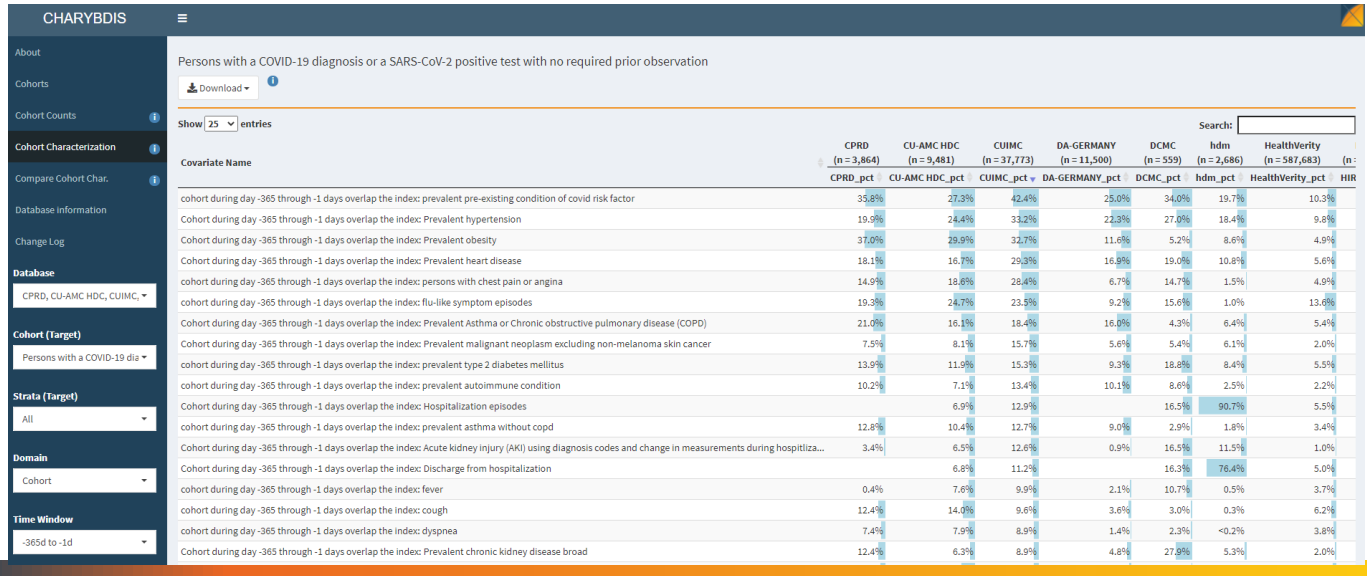


Understand clinical context





https://data.ohdsi.org/Covid19CharacterizationCharybdis/

Expanding the proactive use of real-world evidence for understanding clinical context

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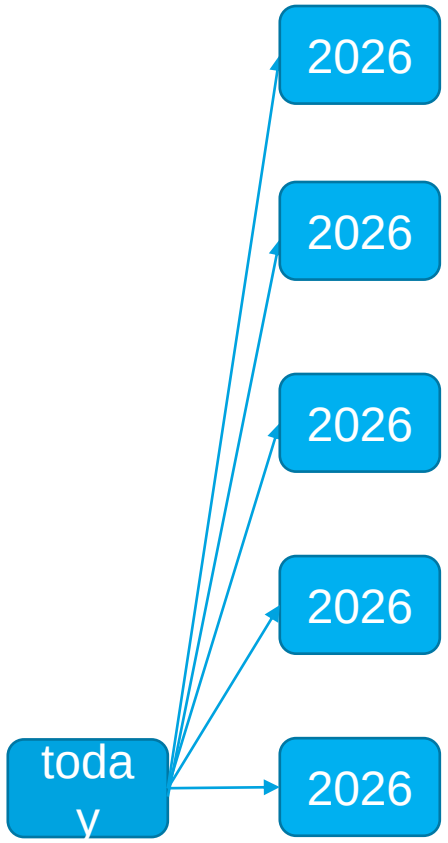

Understand clinical context

thebmj

RESEARCH: SPECIAL PAPER

OPEN ACCESS

Check for updates

FAST TRACK

Characterising the background incidence rates of adverse events of special interest for covid-19 vaccines in eight countries: multinational network cohort study

For numbered affiliations see end of the article.

Correspondence to:
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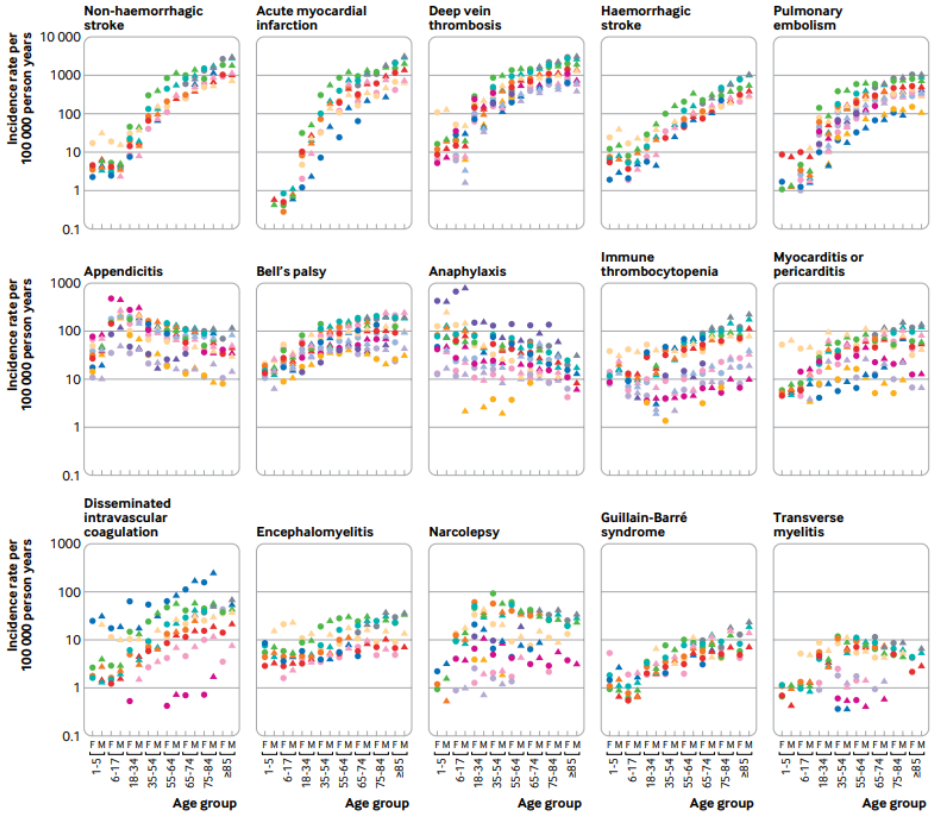
ORCID 0000-0002-3950-6346)

Additional material is published online only. To view please visit the journal online.

Cite this as: *BMJ* 2021;373:n1435
http://dx.doi.org/10.1136/bmj.n1435

Accepted: 3 June 2021



WHAT IS ALREADY KNO

Expanding the proactive use of real-world evidence to investigate associations and impact

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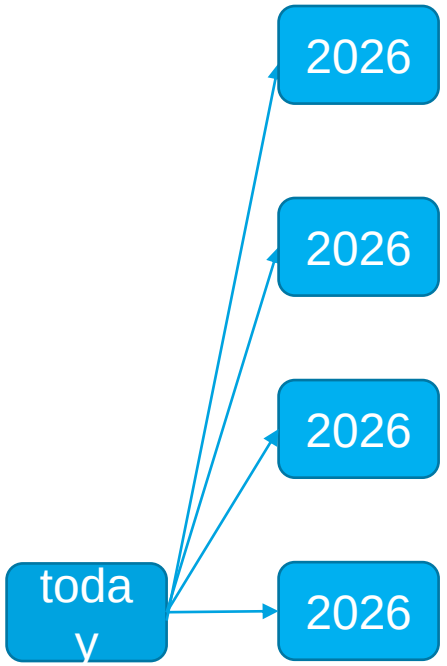


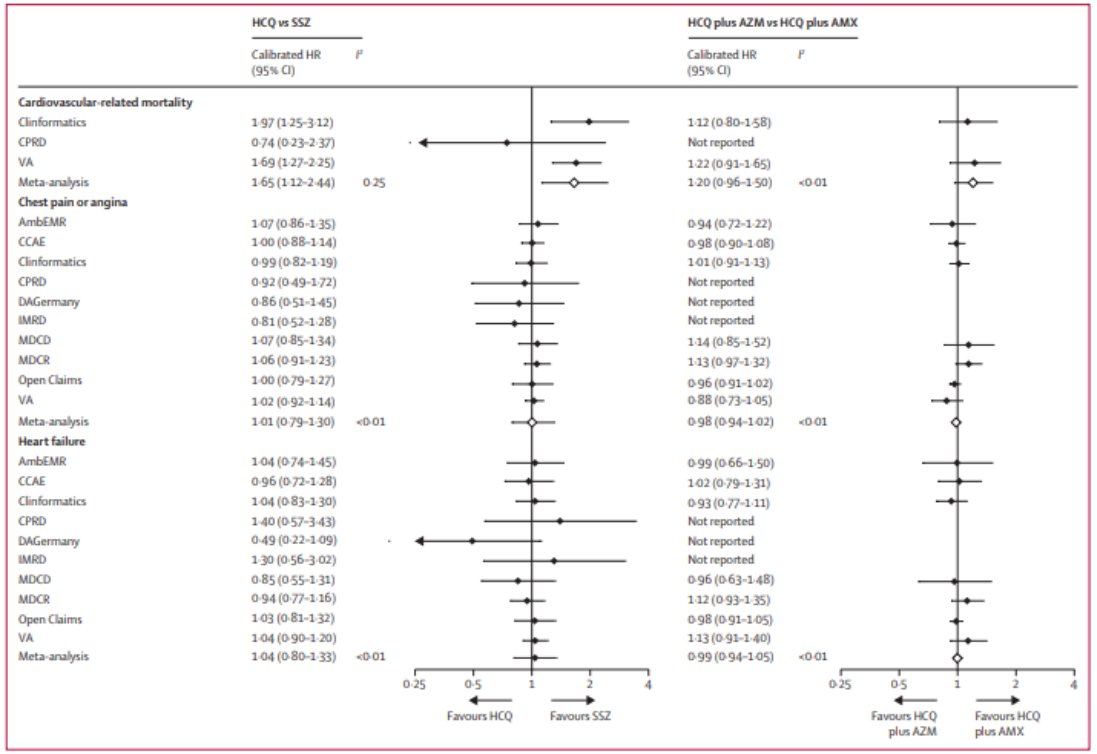
Investigate associations and impact

THE LANCET Rheumatology

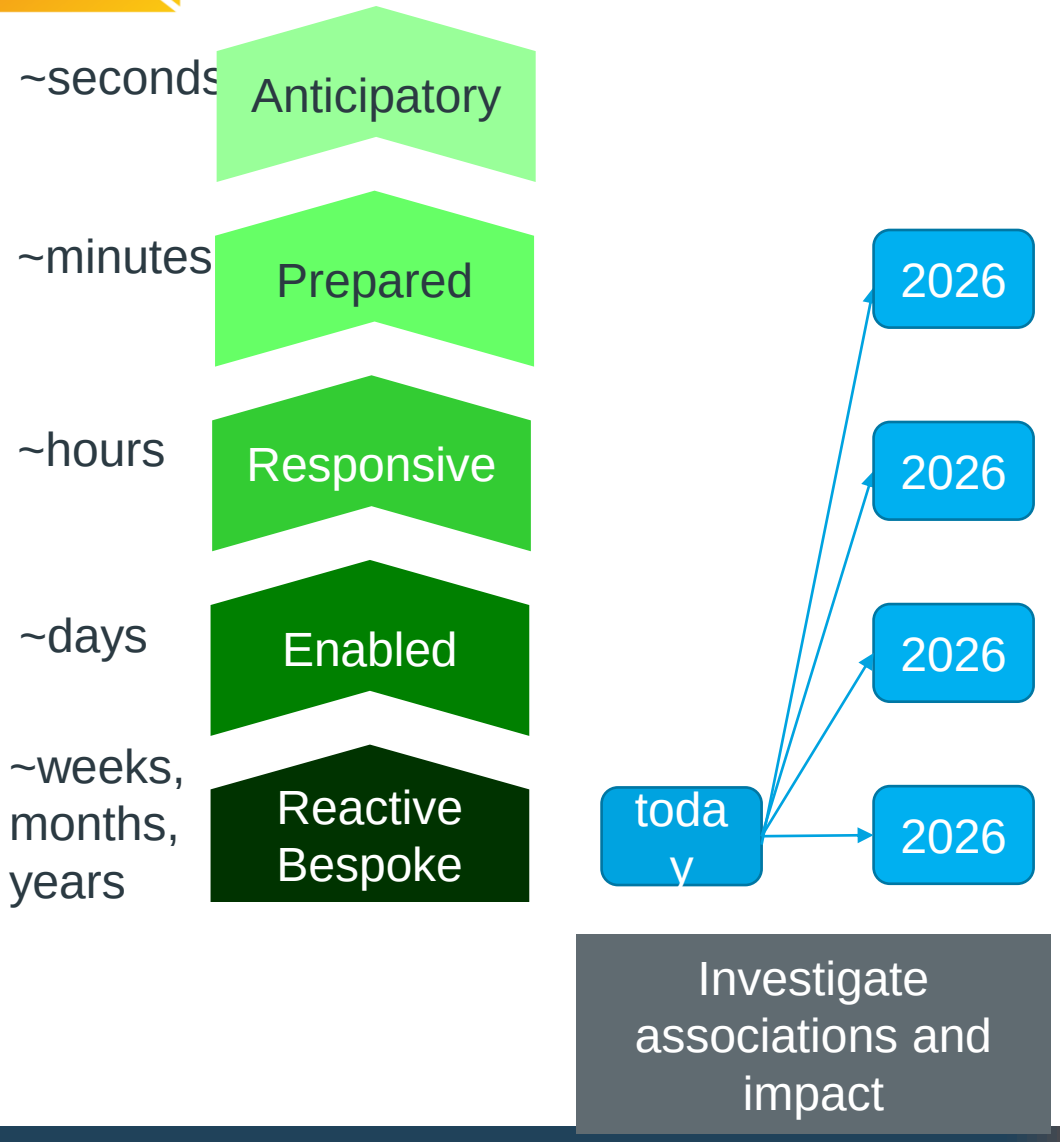
Articles

Risk of hydroxychloroquine alone and in combination with azithromycin in the treatment of rheumatoid arthritis: a multinational, retrospective study

Jennifer C E Lane
Thamir M Alshan
Alexander Davyd
Benjamin Skov K
Rupa Makadia, A
Fredrik Nyberg, A
Selva Muthu Kun
Carmen O Torre, I
Daniel Prieto-Alh



Expanding the proactive use of real-world evidence to investigate associations and impact



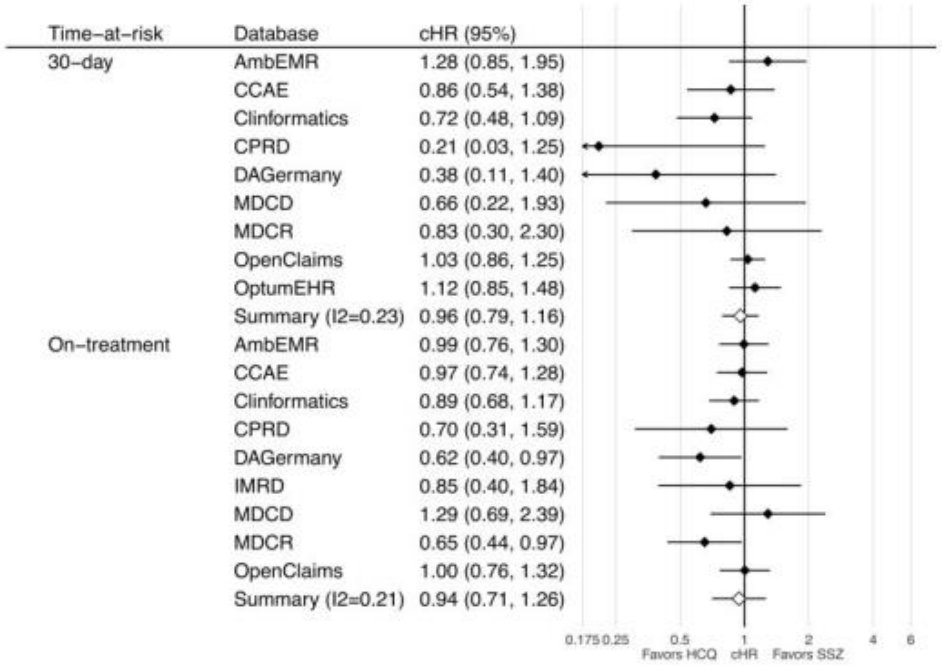
RHEUMATOLOGY

Rheumatology 2021;60:3222–3234
doi:10.1093/rheumatology/keaa771
Advance Access publication 25 December 2020

Original article

Risk of depression, suicide and psychosis with hydroxychloroquine treatment for rheumatoid arthritis: a multinational network cohort study

FIG. 1 Forest plot of the association between short- (top) and long-term (bottom) use of HCQ (*vs* SSZ) and risk of depression, by database and in the meta-analysis



Expanding the proactive use of real-world evidence to investigate associations and impact

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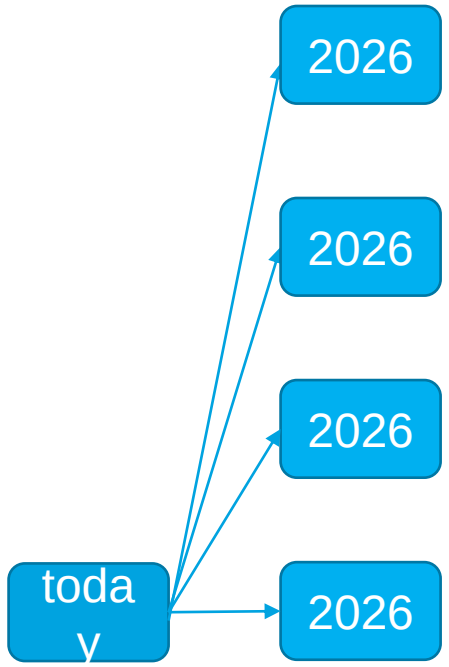
~days **Enabled**

~weeks, months, years **Reactive Bespoke**



Investigate associations and impact

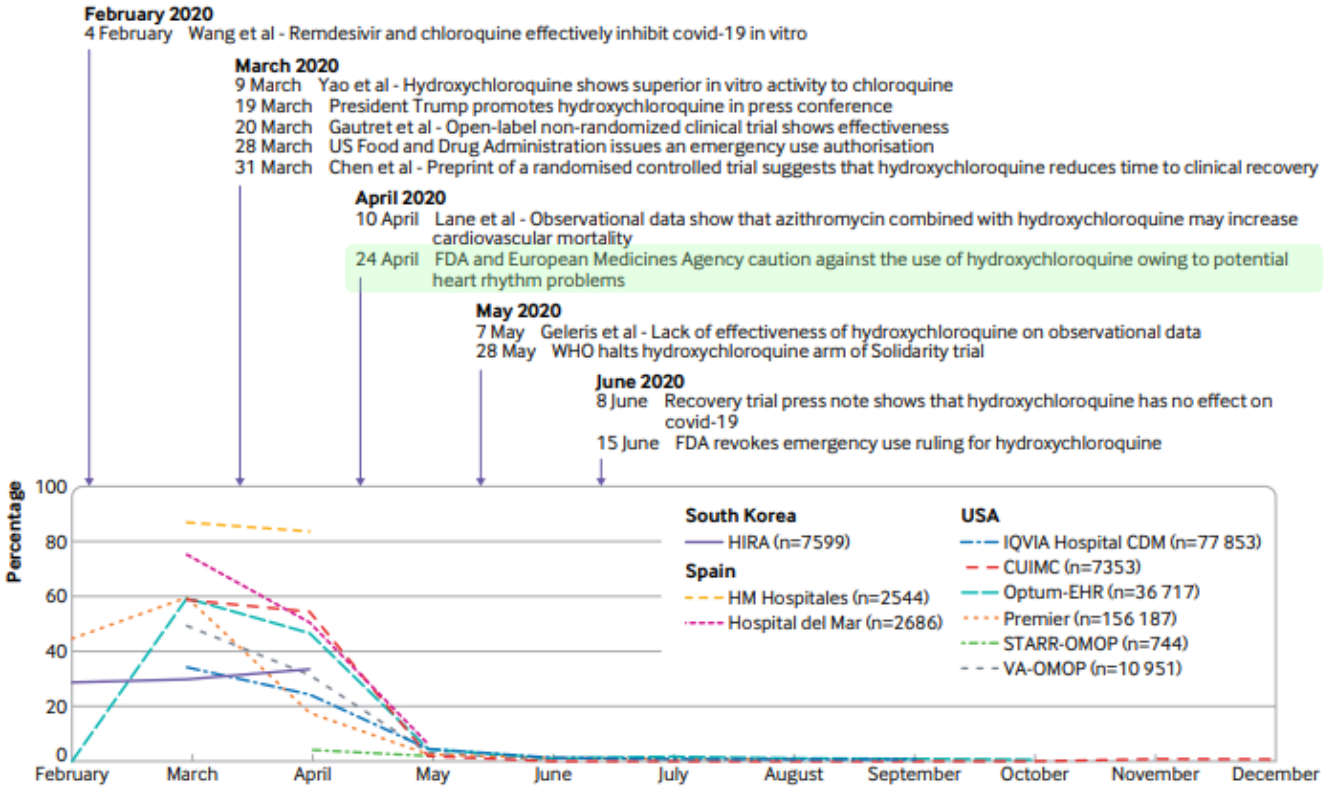
thebmj RESEARCH

OPEN ACCESS

Check for updates

Use of repurposed and adjuvant drugs in hospital patients with covid-19: multinational network cohort study

Albert Prats-Uribe,[1] Anthony G Sena,[2,3] Lana Yin Hui Lai,[4] Waheed-Ul-Rahman Ahmed,[5,6] Heba Alghoul,[7] Osaid Alser,[8] Thamir M Alshammari,[9] Carlos Areia,[10] William Carter,[11] Paula Casajust,[12] Dalia Dawoud,[13,14] Asieh Golozar,[15,16] Jitendra Jonnagaddala,[17] Paras P Mehta,[18] Mengchun Gong,[19] Daniel R Morales,[20,21] Fredrik Nyberg,[22] Jose D Posada,[23] Martina Recalde,[24,25] Elena Roel,[24,25] Karishma Shah,[5] Nigam H Shah,[23] Lisa M Schilling,[11]



Concluding thoughts

- Enabling use and establishing value of real-world evidence requires building trust across stakeholders – evidence generators and consumers
- People and processes need to be augmented with science, technology and engineering
  - Research network = people + data + analytic tools + best practices
- Open science systems that promote transparency and reproducibility can increase reliability and efficiency
- Regulatory use cases are primarily characterization analyses, have been demonstrated to be feasible, and are ready-to-scale
- Community efforts today can enable a more proactive future tomorrow
  - Design of standardized outputs for regulatory use cases
  - Standardized analytic tool development
  - Phenotype development and evaluation

Introducing the Master of Science in Real World Evidence in Healthcare and Life Sciences at Northeastern University Bouvé College of Health Sciences

- Professional-level masters to upskill and meet the workforce demands of Life Sciences and Healthcare companies
- Industry-focused
- Experiential learning with the OHDSI Lab, a cloud-based learning environment with real data
- World-class faculty from leading RWE communities (ICPE, OHDSI)

Partner with Us!

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Contact: Kristin Kostka
Acting Program Director
Director of the OHDSI Center
rwe@northeastern.edu

Program Details

Location: Boston Campus, Online, Hybrid
Commitment: Full-time, Part-time
Enrollment Deadline: Rolling
Deadline for Spring 2023 Start: Dec 1, 2022

Program Credit/GPA Requirements:
- 31 total semester hours required
- Minimum 3.000 GPA required

Curriculum

Core courses:
- HSCI 5130: Introduction to Real World Evidence
- HSCI 5140: Foundations of Data Models
- HSCI 5150: Methods for Observational Research 1
- HSCI 5160: Standardization of Real World Data
- HSCI 5170: Data Model Transformation
- HSCI 5151: Methods for Observational Research 2
- PHSC 5252: Research Skills and Ethics
- HSCI 6980: RWE Capstone

Selectives in Cohort Building / Phenotyping, Advanced Methods

The Roux Institute at Northeastern University

Ensuring Impact and Value of Medical Research to Patients

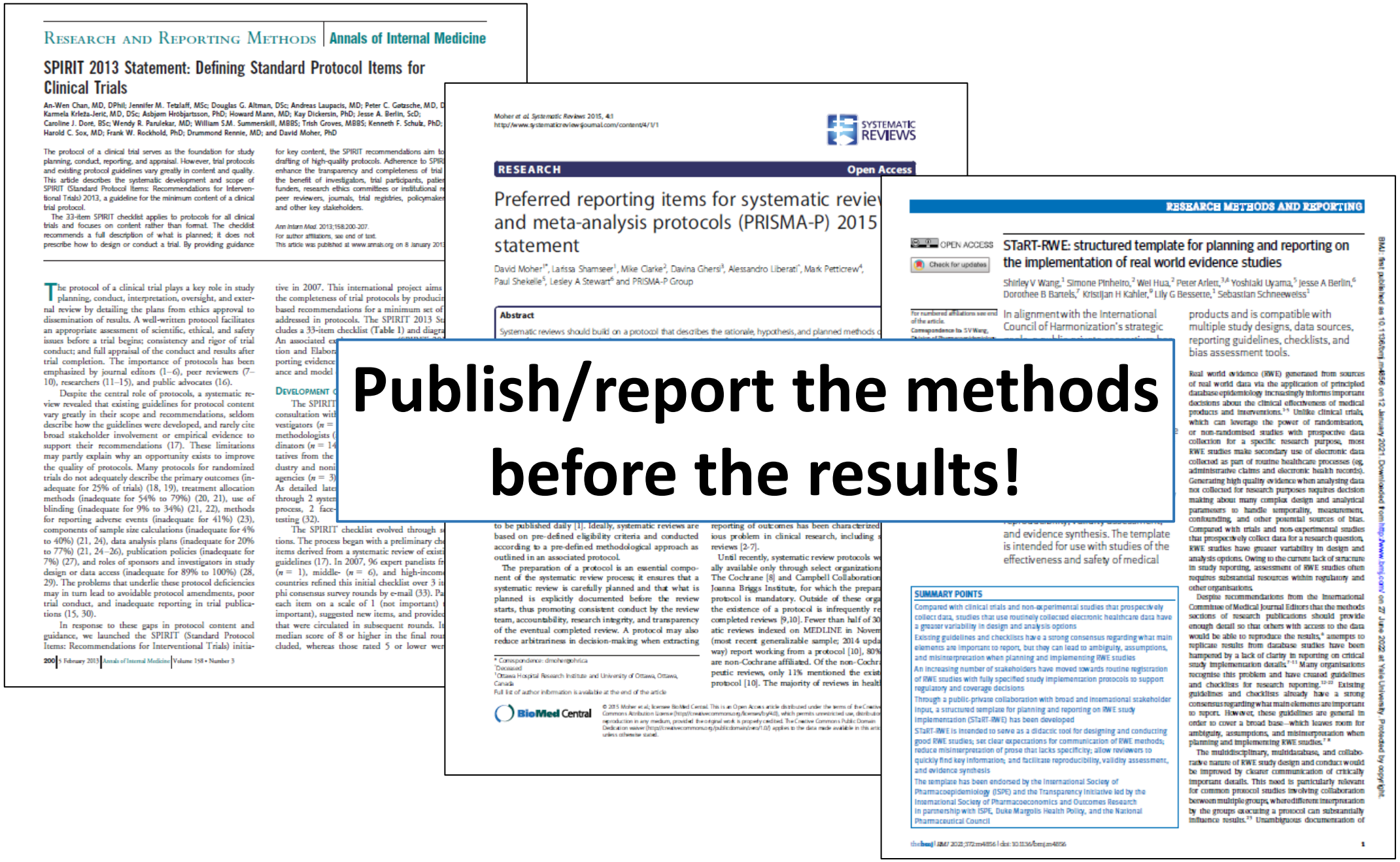
IQVIA Research Forum

October 12, 2022

Joseph S. Ross, MD, MHS
Section of General Internal Medicine, Yale School of Medicine
Associate Editor, The BMJ Co-Founder, medRxiv
@jsross119

Critical considerations for ensuring research impact and value to patients

- **Bring clinical experience to the table**
- **Engage patients and policy makers to establish the research question**
- **Publicly pre-specify study design, endpoints, methods**
- **Timely and comprehensive results reporting**
- **Translate your findings for patients**
- **Support others in the research community**

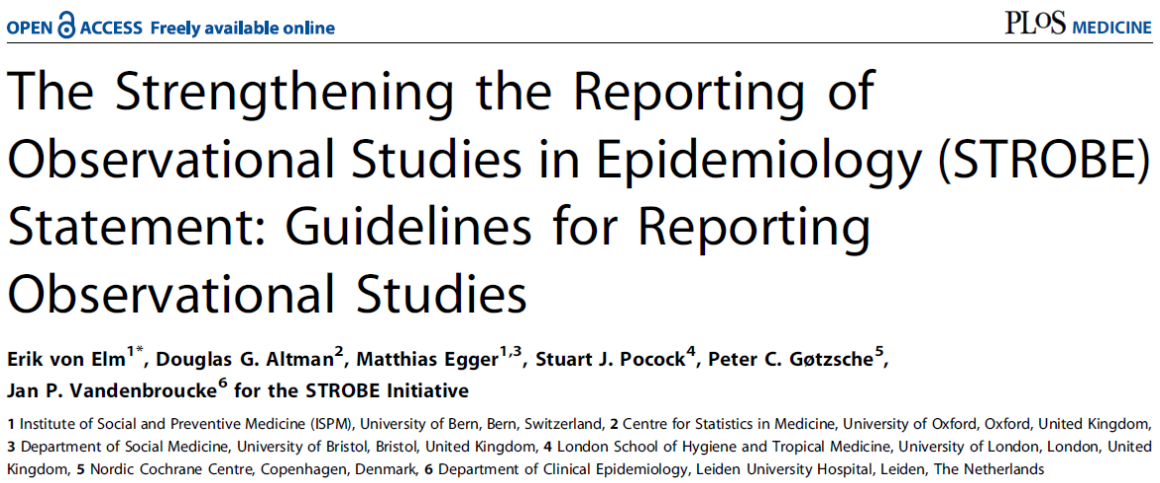


Publish/report the methods before the results!

Source: Ann Intern Med 2013;158:200-207. Sys Reviews 2015;4:1. BMJ 2021;372:m4856.

Timely and comprehensive results reporting

- **Research projects invariably deviate from the pre-specified study protocol and analysis plan – report everything that you said you would do that you did and explain everything that you did not!**
- **Follow EQUATOR guidelines on research reporting**
- **Clinical trial registration number and/or link to study protocol**



OPEN ACCESS Freely available online PLOS MEDICINE

The Strengthening the Reporting of Observational Studies in Epidemiology (STROBE) Statement: Guidelines for Reporting Observational Studies

Erik von Elm[1*], Douglas G. Altman[2], Matthias Egger[1,3], Stuart J. Pocock[4], Peter C. Gøtzsche[5], Jan P. Vandenbroucke[6] for the STROBE Initiative

1 Institute of Social and Preventive Medicine (ISPM), University of Bern, Bern, Switzerland, 2 Centre for Statistics in Medicine, University of Oxford, Oxford, United Kingdom, 3 Department of Social Medicine, University of Bristol, Bristol, United Kingdom, 4 London School of Hygiene and Tropical Medicine, University of London, London, United Kingdom, 5 Nordic Cochrane Centre, Copenhagen, Denmark, 6 Department of Clinical Epidemiology, Leiden University Hospital, Leiden, The Netherlands

RESEARCH METHODS AND REPORTING

OPEN ACCESS

Check for updates

The reporting of studies conducted using observational routinely collected health data statement for pharmacoepidemiology (RECORD-PE)

Sinéad M Langan,[1] Sigrún AJ Schmidt,[2] Kevin Wing,[1] Vera Ehrenstein,[2] Stuart G Nicholls,[3,4] Kristian B Filion,[5,6] Astrid Guttmann,[10] Sebastian Schneew Shirley V Wang,[14] Er

GUIDELINE Open Access

Check for updates

Consolidated Health Economic Evaluation Reporting Standards 2022 (CHEERS 2022) statement: updated reporting guidance for health economic evaluations

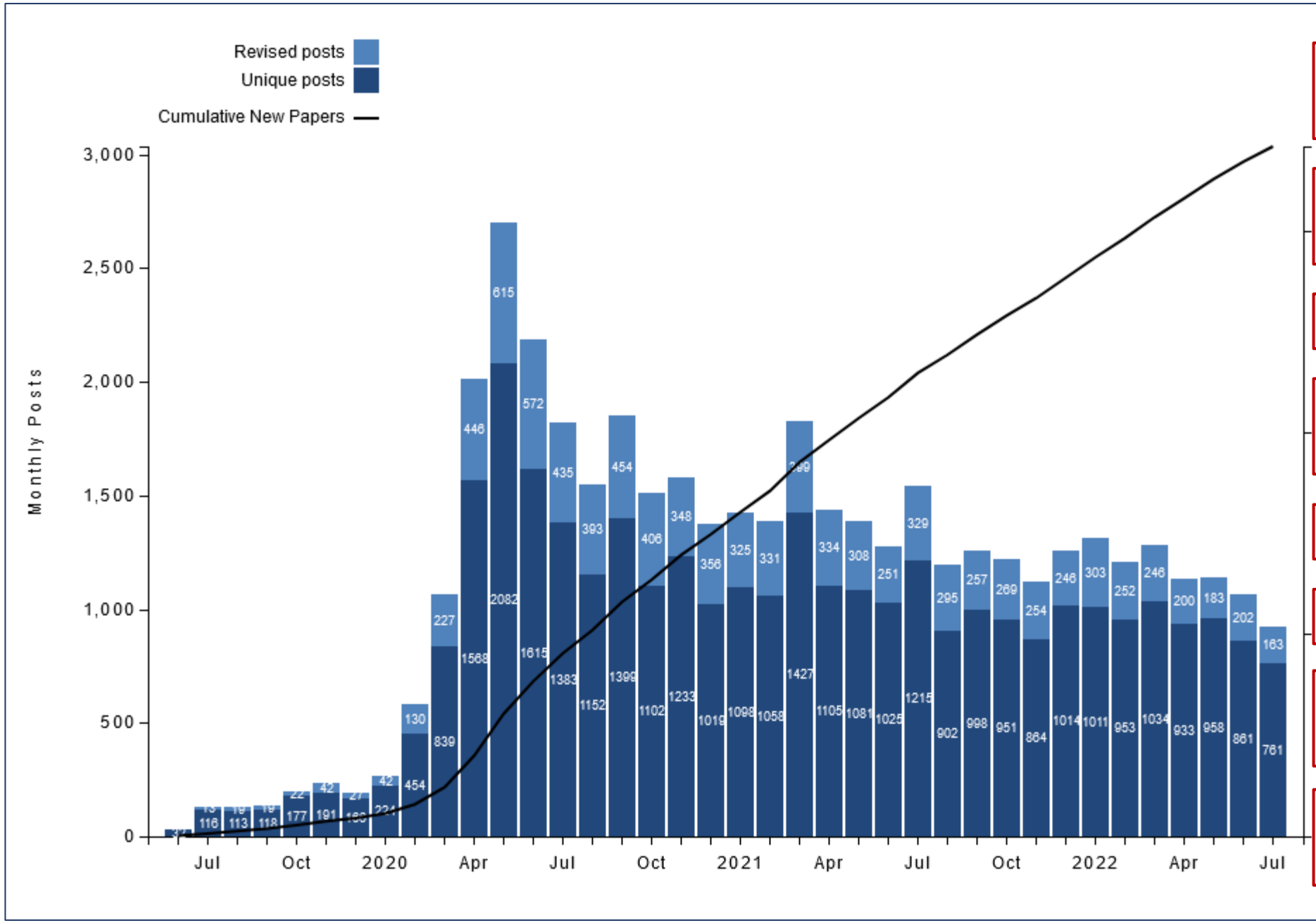
Don Husereau[1,2*], Michael Drummond[3], Federico Augustovski[4,5,6], Esther de Bekker-Grob[7], Andrew H. Briggs[8], Chris Carswell[9], Lisa Caulley[10,11,12], Nathorn Chaiyakunapruk[13], Dan Greenberg[14], Elizabeth Loder[15,16], Josephine Mauskopf[17], C. Daniel Mullins[18], Stavros Petrou[19], Raoh-Fang Pwu[20], Sophie Staniszewska[21] and on behalf of CHEERS 2022 ISPOR Good Research Practices Task Force

Platform Policies

- Submission requirements that follow ICMJE guidance on author details, funding statements, ethical oversight, trial registration
- Only original research articles and study protocols allowed
- Screening by staff and affiliates before posting
- Signals for caution

Potential Benefits

- Rapid, early sharing of new information
- Demonstrates scientific productivity
- Prompts scientific feedback and enhances collaboration



~41k unique articles submitted

~34k unique articles posted

~83% posted

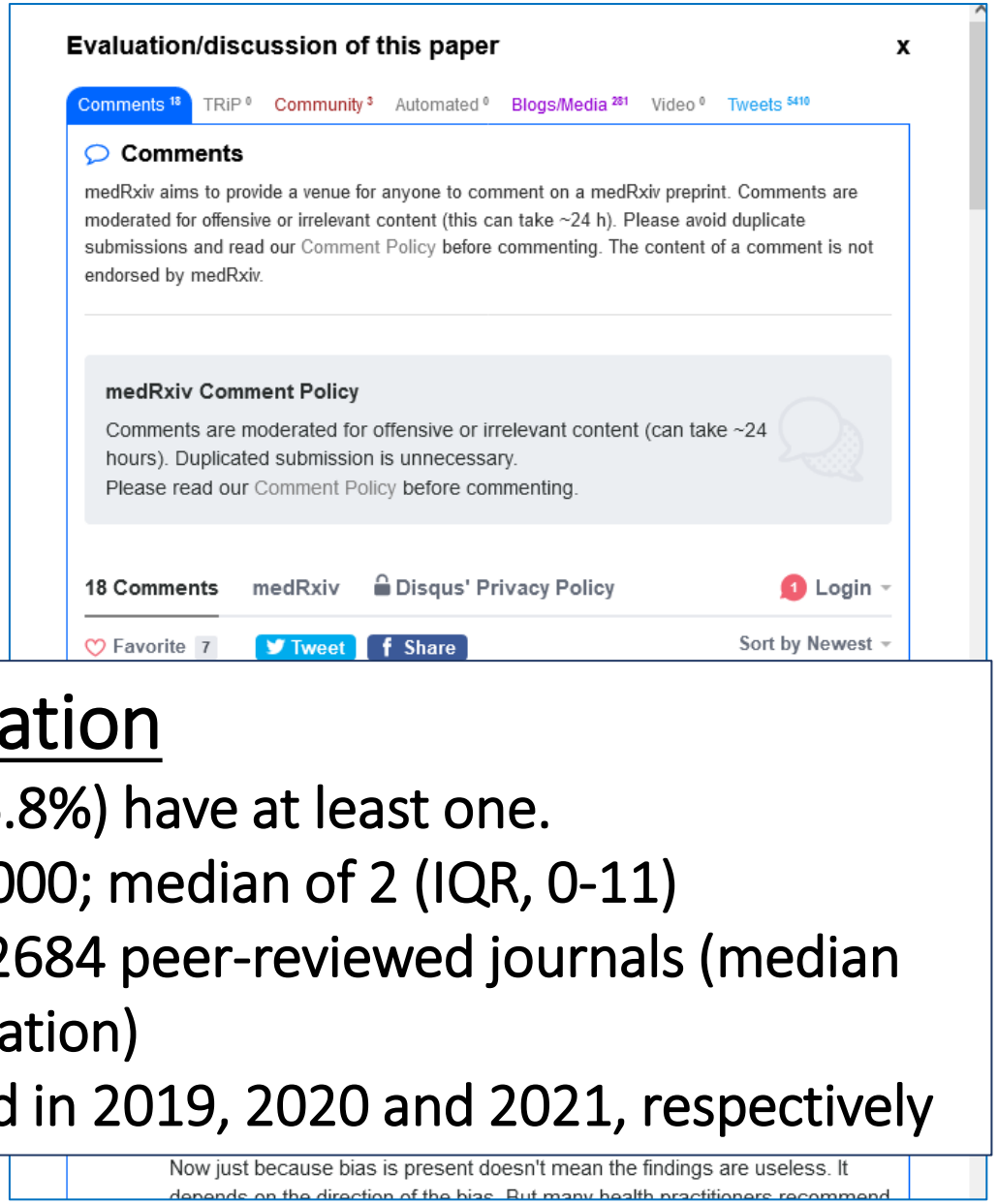
~185k unique authors

~155 countries

~21% revised ≥1x

56 (0.16%) withdrawn

~54% COVID-19 related



Community Engagement and Dissemination

- 8754 total user comments on preprints; 2352 (6.8%) have at least one.
- 312 (1.1%) preprints with an Altmetric score >1000; median of 2 (IQR, 0-11)
- Thus far, 13,361 (38.8%) preprints published in 2684 peer-reviewed journals (median interval of 153 days between posting and publication)
  - 63.0%, 48.2%, and 22.5% of preprints posted in 2019, 2020 and 2021, respectively

Translate findi

WHAT IS ALREADY KNOWN ON THIS TOPIC?

Evidence on the links of infertility, miscarriage, and stillbirt
been inconclusive

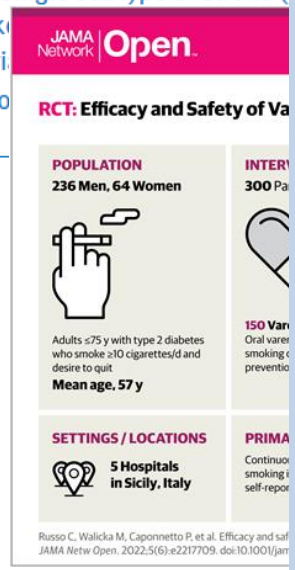
Limited evidence is available on the association of infertili
stillbirth with stroke by subtype

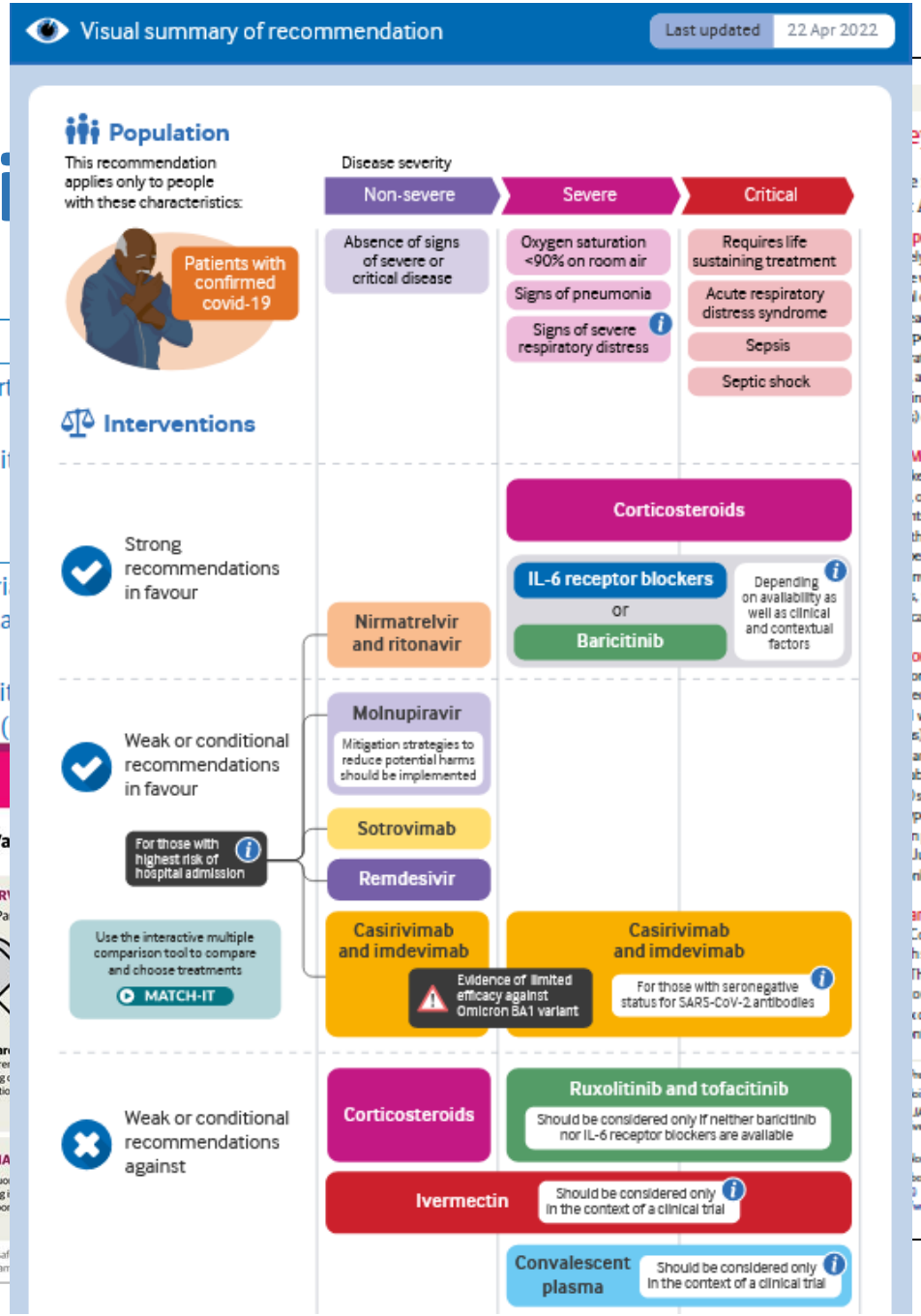
WHAT THIS STUDY ADDS

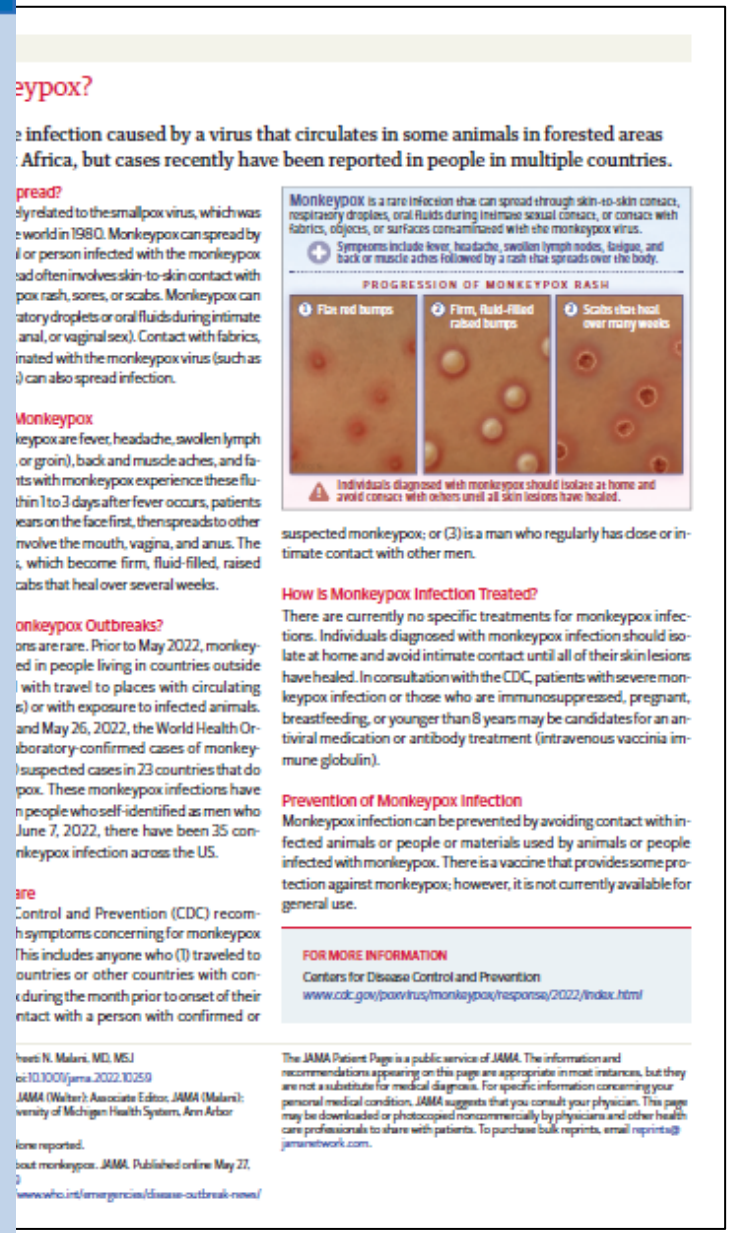
Infertility and pregnancy loss, especially recurrent miscarri
and recurrent stillbirth (at least two), increased women's la
and fatal stroke

The risk of non-fatal or fatal stroke associated with infertili
stillbirths was mainly driven by a single subtype of stroke (
stroke or fatal haemorrhagic strok
associated with recurrent miscarri

A history of recurrent pregnancy lo
factor for stroke







Source: BMJ 2022;377:e070603. JAMA Network Open 2022;5:e2217709. JAMA 2022;online first. BMJ 2020;370:m3379.

Strengthening Science through Data Sharing

- Ensures all data can be used to inform clinical decisions
- Positions research as a public good
- Respects contributions of participants:
  - maximizing value of collected data, while
  - minimizing duplicative data collection
- Facilitates secondary studies of existing data
- Promotes transparency and reproducibility:
  - sample, design, and analysis

Q&A

Post your questions and comments in the Q&A box

Charting the future of high impact health research

Panelists

Caleb Alexander, MD, MS
Professor
Johns Hopkins Bloomberg School of Public Health

Mui Van Zandt
VP&GM Real World Data & Tech
IQVIA

Murray Aitken
Executive Director
IQVIA Institute for Human Data Science

Moderator: Stig Albinus, Senior Advisor, IQVIA Institute for Human Data Science

Charting the Future with High Impact Health Research - Perspectives from the 2022 Research Forum

High impact health research is delivered through:

Advancing a multidisciplinary learning health systems approach to national health crises, balancing timeliness and quality

Rethinking research approaches to social determinants of health across a range of issues, including gaps in data, linkage of data and validation of what data matters most

Navigating the complexity and heterogeneity of patient affordability and access in diverse populations by focusing on gaps or missing data, and the impact of the Inflation Reduction Act

Elevating the value to patients of academic health research

2022 Research Forum Agenda

Exploring the elusive nature of applied research in national health crises

How to improve the effectiveness of critical health research?

Monday, Oct 10
10 - 11 a.m.

Incorporating the impact of social determinants on outcomes in healthcare research

What are the considerations for the broader use of social determinants in research?

Monday, Oct 10
11 a.m. - 12 p.m.

Navigating the complexity and heterogeneity of patient affordability and access

What do affordability and access mean in a diverse population?

Tuesday, Oct 11
10 - 11 a.m.

Elevating the value to patients of academic health research

What research strategies can achieve increased value to patients?

Wednesday, Oct 12
10 - 11 a.m.

Charting the future of high impact health research

How to raise the ongoing value of health research?

Wednesday, Oct 12
11 a.m. – 12 p.m.

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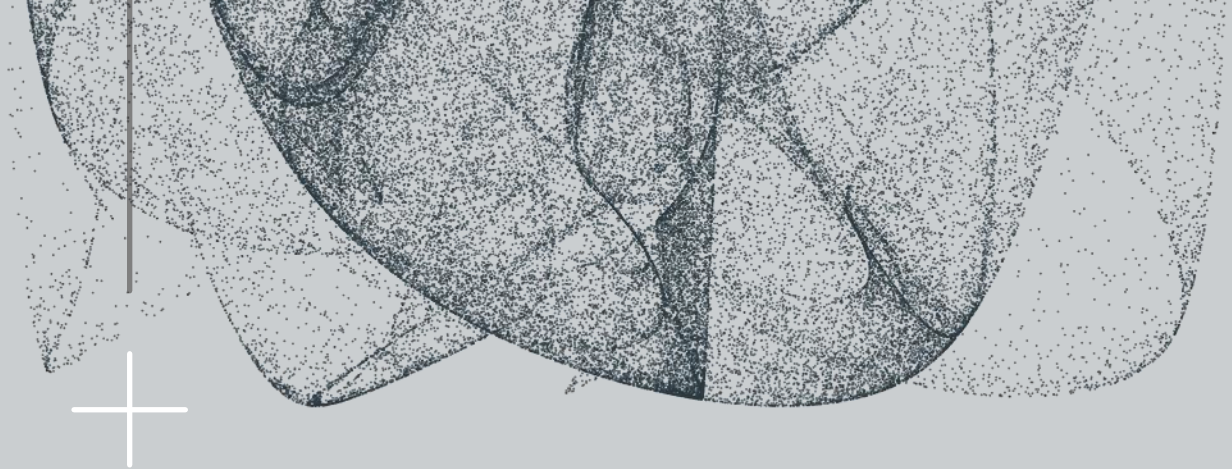

IQVIA INSTITUTE FOR HUMAN DATA SCIENCE

Thank you