



# Making the Most of Real-World Data

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- C-Path is a public-private partnership with US FDA.
- Speaker receives funding from FDA, CDC, and NIH.
- Content of this presentation is not intended to represent endorsement or approval from any organization or government entity.

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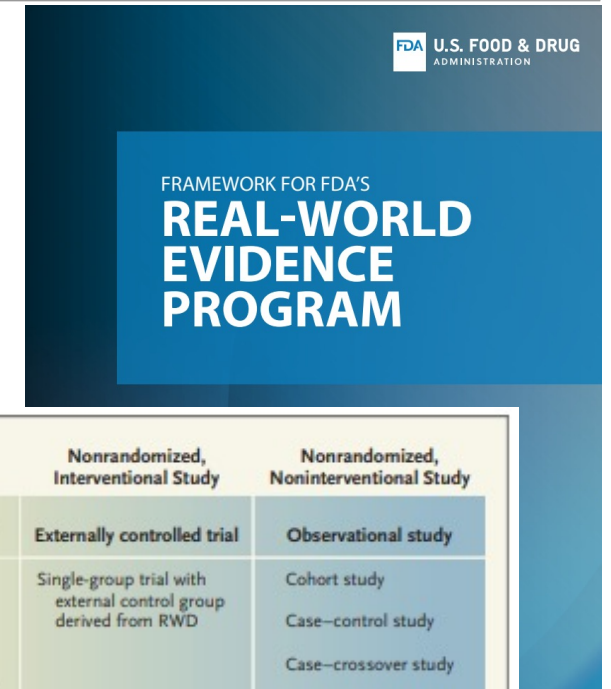
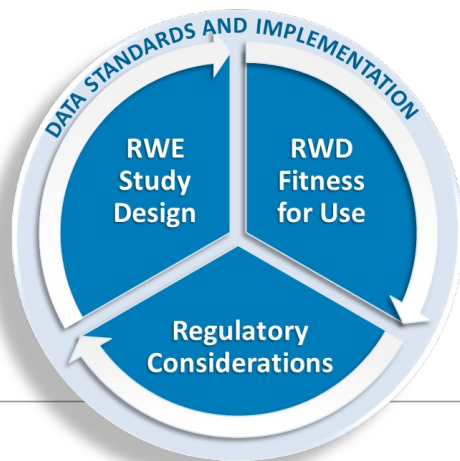


- Drug Repurposing
- Real-World Data
- The Edge Tool
  - Use Case: COVID-19
- Real-World Evidence
- Future Directions

- Significant portion of conditions have no FDA approved treatments
- Traditional drug development and labeling is slow and expensive.
- Repurposing may be needed in diseases which are:
  - rapidly emerging
  - extremely rare
  - impacting vulnerable groups
  - treated by standardized guidelines



- Explore clinical practice
- Develop and refine hypotheses
- Provide external controls
- Observational research



Randomized, Interventional Study		Nonrandomized, Interventional Study	Nonrandomized, Noninterventional Study
Traditional randomized trial using RWD in planning	Trial in clinical practice settings, with pragmatic elements	Externally controlled trial	Observational study
RWD used to assess enrollment criteria and trial feasibility RWD used to support selection of trial sites	Selected outcomes identified using, e.g., health records data, claims data, or data from digital health technologies  RCT conducted using, e.g., electronic case report forms for health records data or claims data	Single-group trial with external control group derived from RWD	Cohort study Case-control study Case-crossover study
Generation of RWE			
Increasing reliance on RWD			

Reliance on RWD in Representative Types of Study Design.

RCT denotes randomized, controlled trial; RWD real-world data; and RWE real-world evidence.

Concato J, Corrigan-Curay J. Real-World Evidence — Where Are We Now? *N Engl J Med.* 2022;386(18):1680-1682. doi:10.1056/NEJMp2200089

- Unstructured data
- Defining outcomes
- Privacy concerns
- Loss to follow up
- Data harmonization

- Imagine we instructed everyone in this meeting to collect the eye color, hair color, height, and coffee preferences of people at the grocery store.
- How would you store the data?
- What variable names would you use?
- How would you classify responses?
- Would you code responses? Free text?

Eye_Color	Hair_Color	Height	Coff_Pref
Brown	Blonde	5ft6in	Black
Blue	Brown	5ft8in	Cream
Brown	Grey	4ft11in	Just sugar
Hazel	Brunette	6ft1in	Oat milk
Brown	Red	5ft11	Iced
Green	Dirty Blonde	5ft9in	Tea only

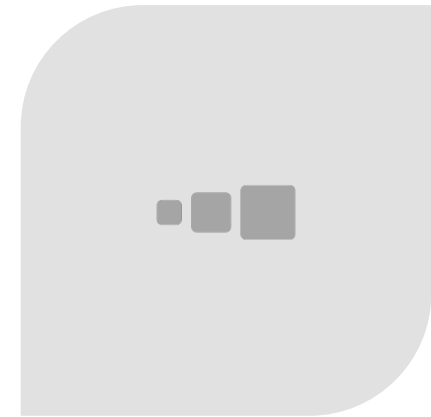




**EXTRACT**



**TRANSFORM**



**LOAD**

**ETL**

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- Centralized harmonization
- Sites use common data model at baseline
- Only for US data
- Limited to COVID-19

## National COVID Cohort Collaborative (N3C)

The N3C is a partnership among the NCATS-supported [Clinical and Translational Science Awards \(CTSA\) Program](#) hubs, the [National Center for Data to Health \(CD2H\)](#), and NIGMS-supported [Institutional Development Award Networks for Clinical and Translational Research \(IDeA-CTR\)](#), with overall stewardship by NCATS. Collaborators will contribute and use COVID-19 clinical data to answer critical research questions to address the pandemic.



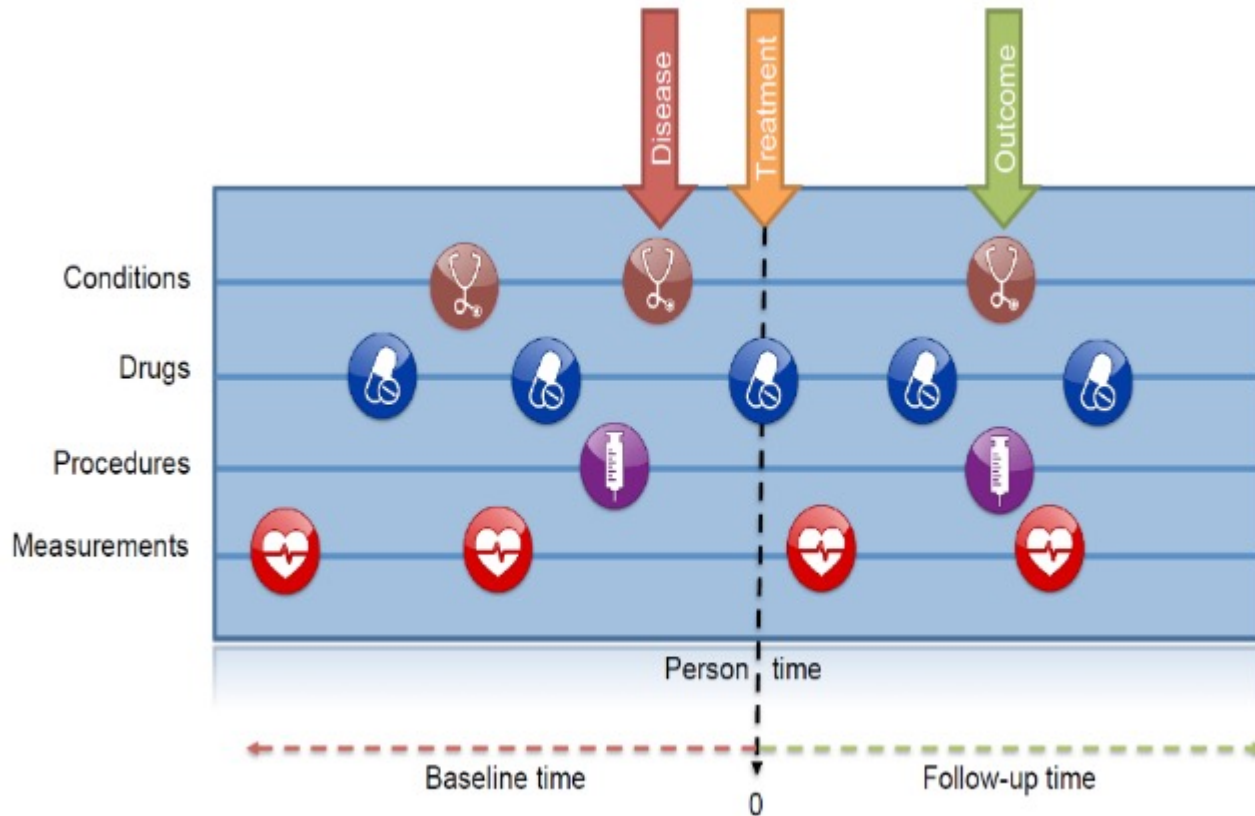
### Scientists Use N3C Data to Identify Common Features of Long COVID

NIH-supported researchers used electronic health record data from the National COVID Cohort Collaborative (N3C) Data Enclave to identify people with long COVID and those likely to have it. [▶](#)

[ncats.nih.gov/n3c](https://ncats.nih.gov/n3c)

# Observational Medical Outcomes Partnership (OMOP)

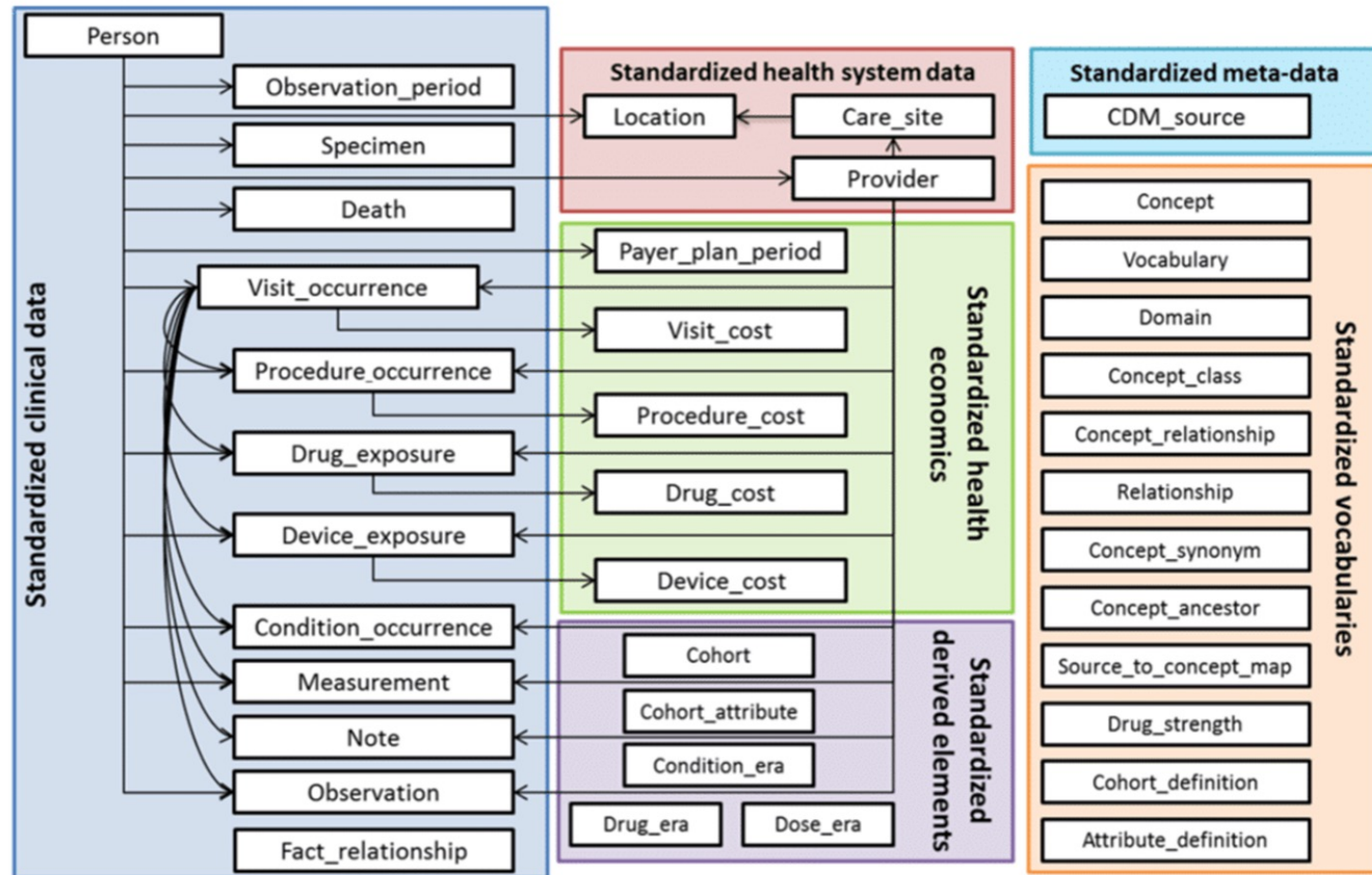
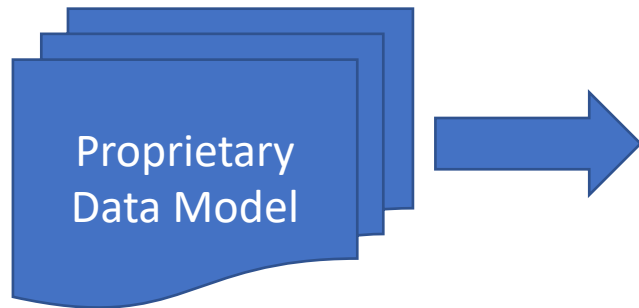
153 Controlled Vocabularies  
9 Million concepts



- Color by First(vocabulary\_id)
- APC
  - ATC
  - Cohort
  - CPT4
  - DRG
  - ETC
  - Gemscript
  - Genseqno
  - GPI
  - HCPCS
  - ICD10
  - ICD10CM
  - ICD9CM
  - ICD9Proc
  - Indication
  - LOINC
  - MDC
  - MedDRA
  - MESH
  - Multilex
  - Multum
  - NDC
  - NDFRT
  - OPCS4
  - OXMIS
  - PCORNet
  - Read
  - RxNorm
  - SMQ



# The Edge Tool



OMOP CDM V5.3.1

- Web-based decision support for concept mapping
  - Base configuration settings for major EMR Vendors.
  - Configuration management documentation tool
  - Inspection Report of DevOps on ETL processes
  - Data Quality Dashboard framework of 3,000+ data quality tests
  - Collaborative cohort subset definition
  - Perform de-identification and submission
  - **All open-source resources**
-

# The Edge Tool on Azure



docker

ETL

- Perseus
- EMR base config
- Usagi
- White Rabbit
- Rabbit in the Hat



docker

DevOps

- Data Quality Dashboard
- Documentation Engine
- Submission extraction
- Change control



docker

Analysis

- Atlas
- WebAPI
- Hades
- R-Studio
- Methods Library

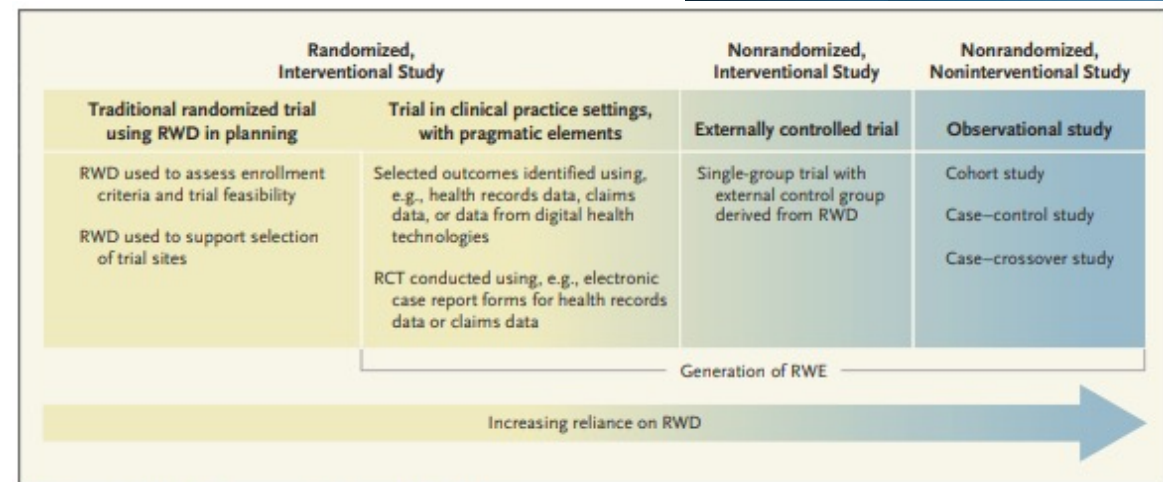
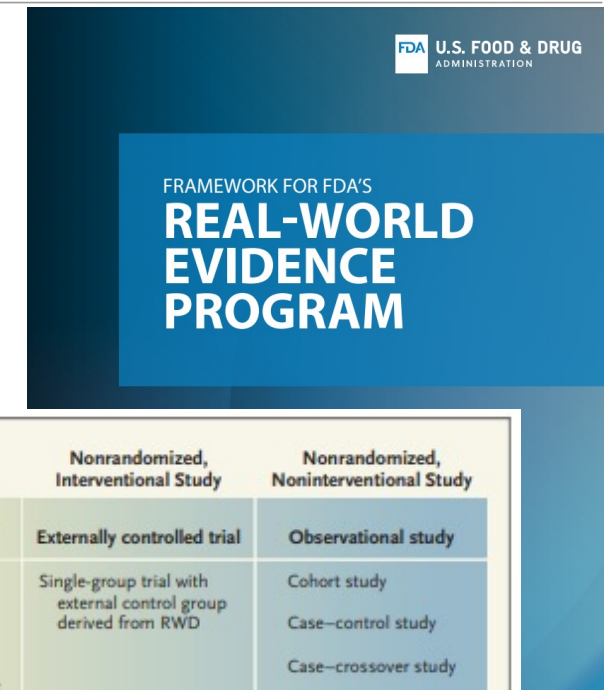


Azure SQL Server  
OMOP Data Model  
Vocabulary Management  
Authentication and Authorization



- Drug Repurposing
- Broad Impact
- Identifiable Cases
- Discreet Data
- Acute Disease
- Definitive Outcomes

- Emulate findings of clinical trials
- Evaluate key agents: dexamethasone, baricitinib, tocilizumab
- Demonstrate utility of Edge Tool

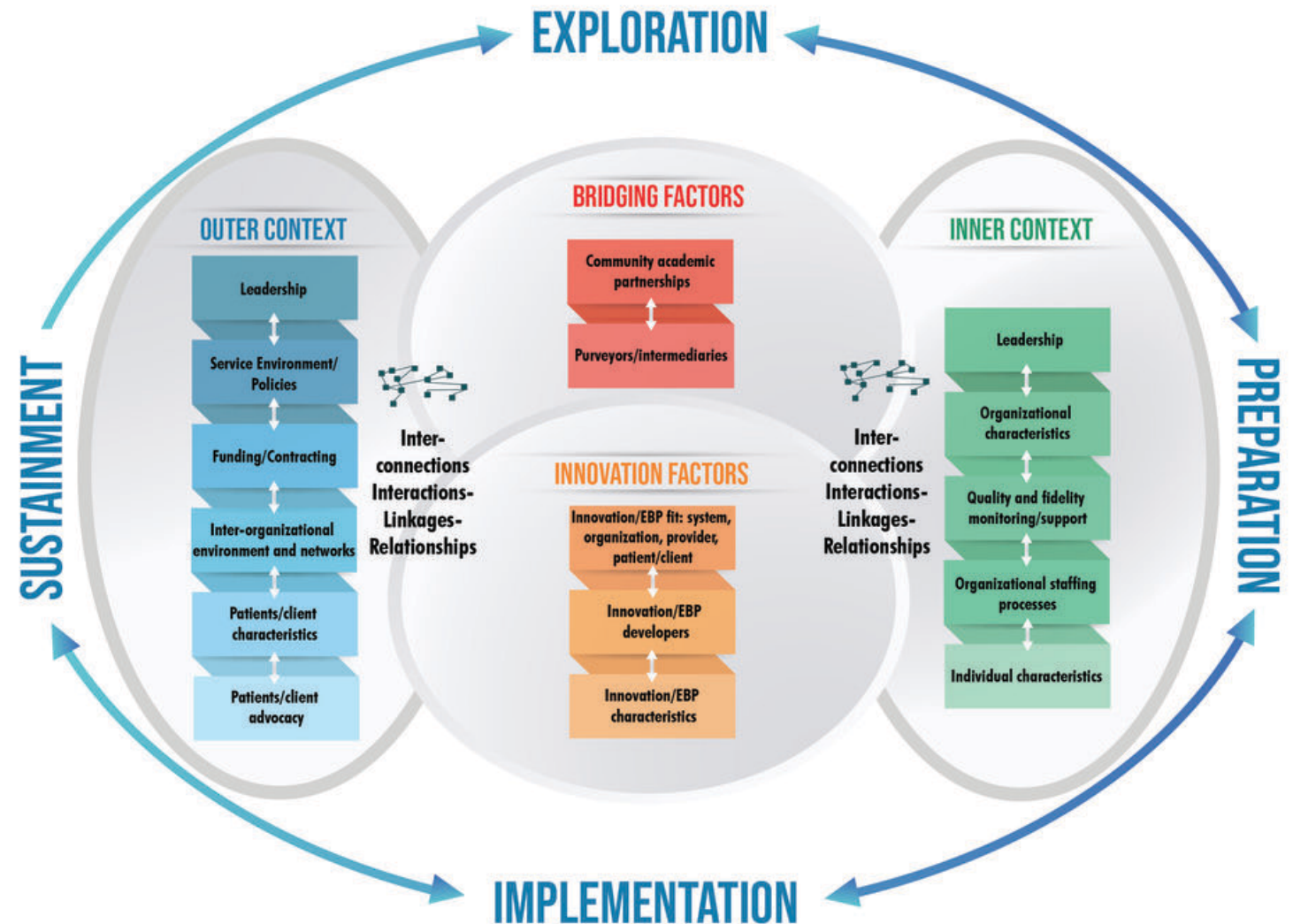


Reliance on RWD in Representative Types of Study Design.

RCT denotes randomized, controlled trial; RWD real-world data; and RWE real-world evidence.

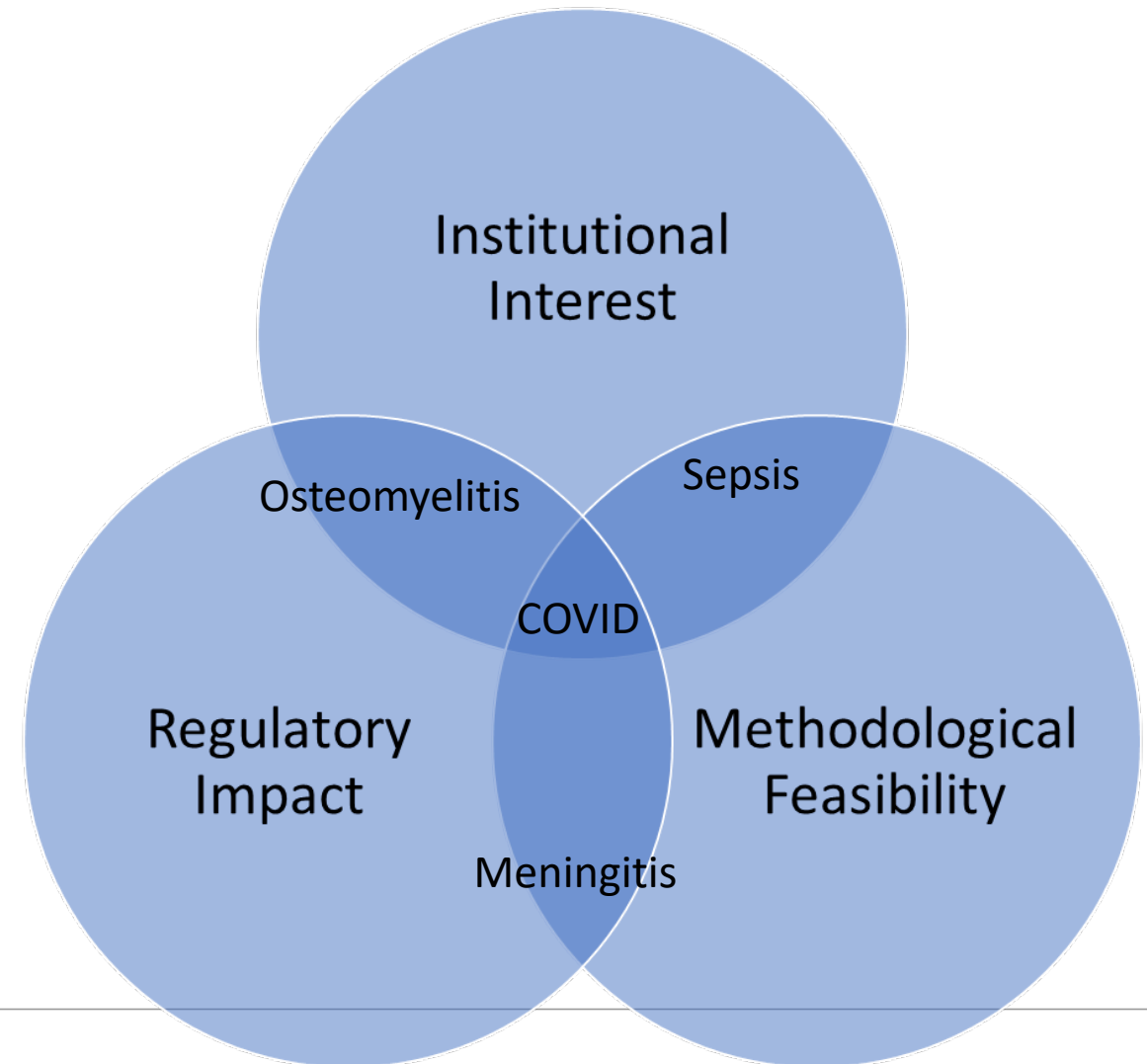
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- What motivates institutions to build infrastructure to extract and share RWD?



<https://episframework.com/>

- Sepsis
  - Isolating organisms
  - Case definition
  - Sites highly motivated
- Meningitis
  - Isolating organisms
  - Rarer cases of more interest
- Osteomyelitis
  - Isolating organisms
  - Linking encounters
  - Lost to follow up





Thank You



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