

The logo for the U.S. Food & Drug Administration, consisting of the letters 'FDA' in white on a blue rectangular background.

**U.S. FOOD & DRUG  
ADMINISTRATION**

CENTER FOR DRUG EVALUATION & RESEARCH

A person's hands are shown holding a tablet computer. The tablet screen displays a blue line graph with a white jagged line on a grid. In the background, there are other data visualizations, including a bar chart and a pie chart, all in shades of blue and white. The overall scene is a professional office setting.

# Real World Data and the PCORTF Common Data Model Harmonization Project

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- Real World Data (RWD)
- Current State and Desired State of Real World Data Access for FDA
- The PCORTF Common Data Model Harmonization for Evidence Generation Project

# Real World Data: Definition



RWD is data on health care that is derived from multiple sources outside typical clinical research settings, including electronic health records (EHRs), administrative claims, product and disease registries, and data gathered through personal devices and health applications

# Real World Data Uses for Clinical Trials



- Rapidly find large numbers of possible participants with all requirements for clinical trial study
  - Health history
  - Treatment history
  - Medicine history
  - Range of demographics
  - Range of geographies and rural/urban
- Rapidly identify site/investigators
- Savings of time and money for manufacturers, clinical investigators, and other stakeholders
- Potential to identify additional “off-label” uses for approved drugs

## Current State for Real World Data

- Thousands of real-world data systems (EHRs, claims data, etc.)
  - Limited data connection to FDA research systems
- Can not effectively conduct large scale real world evidence research

## Ideal State for Real World Data

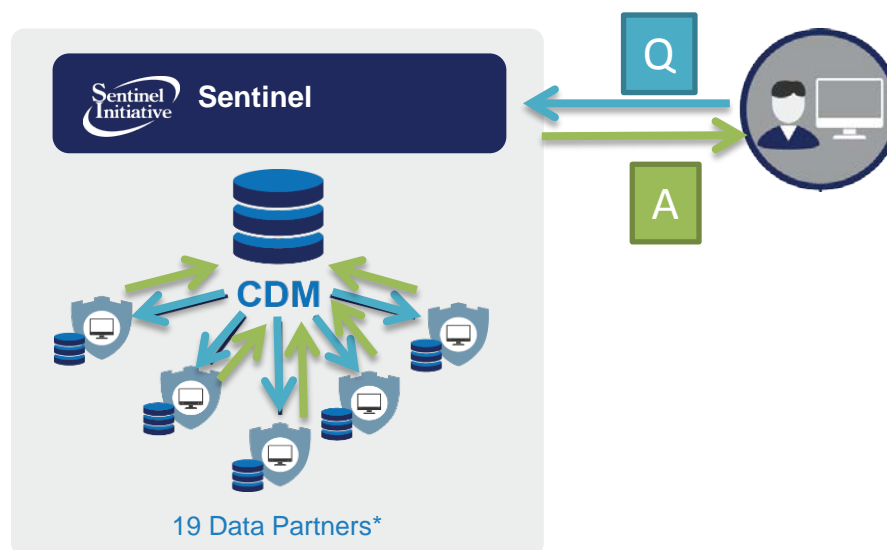
- Real-time access to real world data systems accessible to FDA researchers:
  - Secure
  - Privacy protected / de-identified
  - sources retain ownership and control of raw data
  - Crosses demographics, geography, etc.
- Mechanisms to get rapid answers to research questions from pools of millions of patients
  - Without risk of any privacy breach
  - Results are de-identified

# Current State: Limited groups of connected Real World Data systems



- Different networks use various sources of healthcare and research data.
- Creation of different mini-networks for research
- Allows researcher to query (ask question) and simultaneously, receive results from many different sources.

BUT: Each network communicates with different agreed upon methods: “Common Data Model”



# The current problem with CDMs using International Travel Adapters



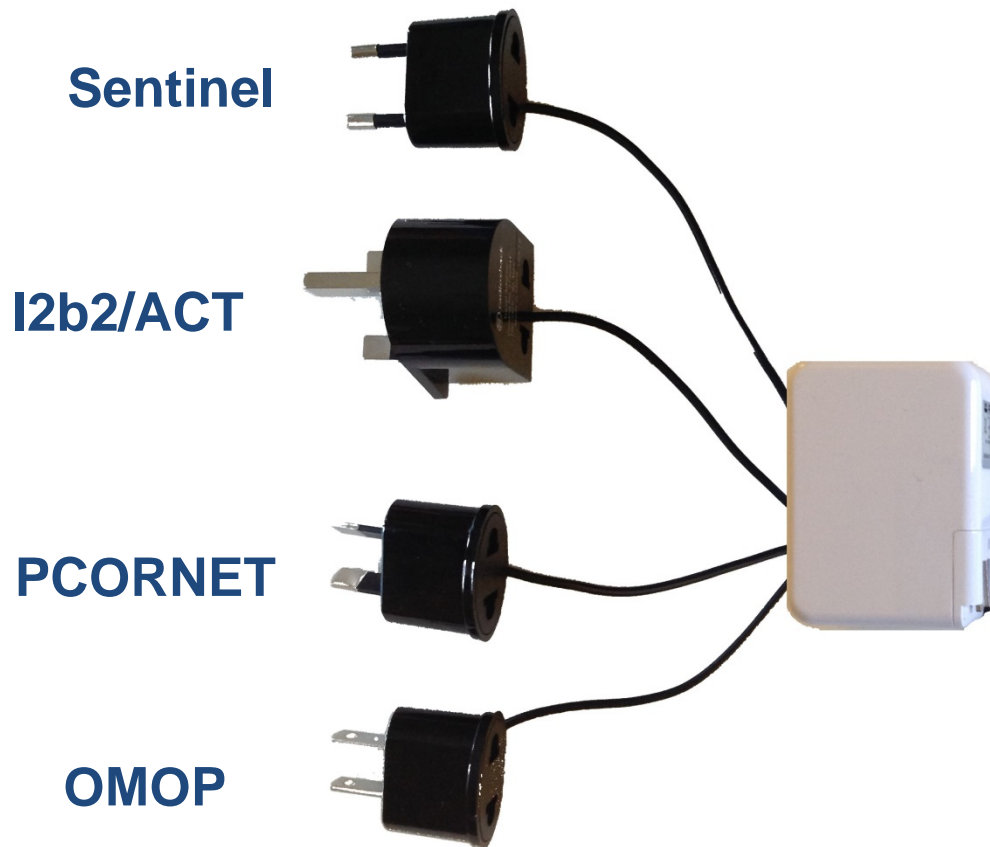
- Different countries use different “outlets”.
- There is a need for travel adapters.

## The Solution:

- Use a converter between various adapters.
- Allow researchers to ask a question once and receive results from many different sources using a common agreed-upon standard structure, or a Common Data Model.



# The solution, using the Adapter Analogy



- Different countries use different “outlets”.
- There is a need for travel adapters.

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# What do we want to do?

## PCORTF CDM Harmonization Project



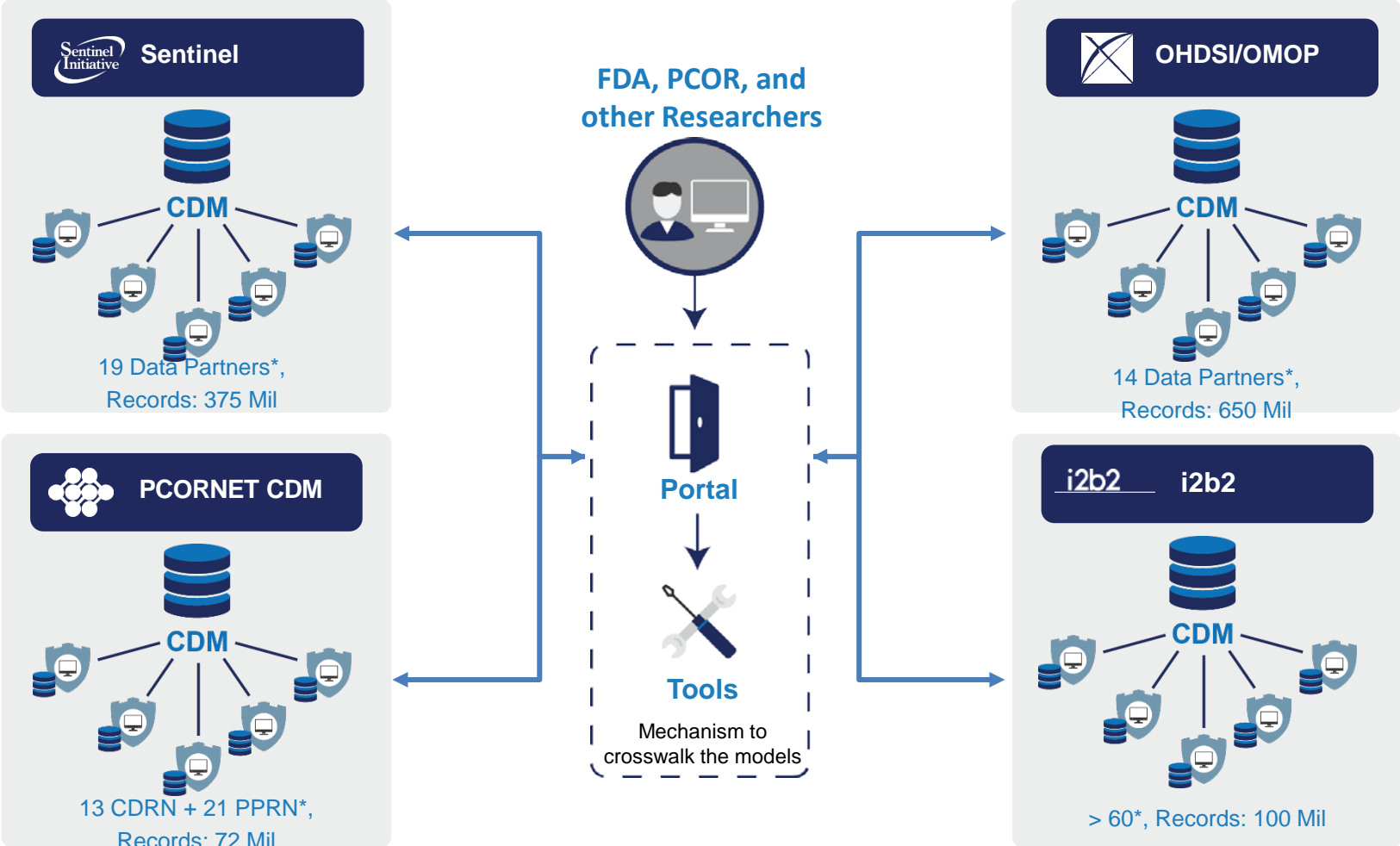
### Goal:

Build a data infrastructure for conducting research using Real World Data derived from the delivery of health care in routine clinical settings.

### Objective:

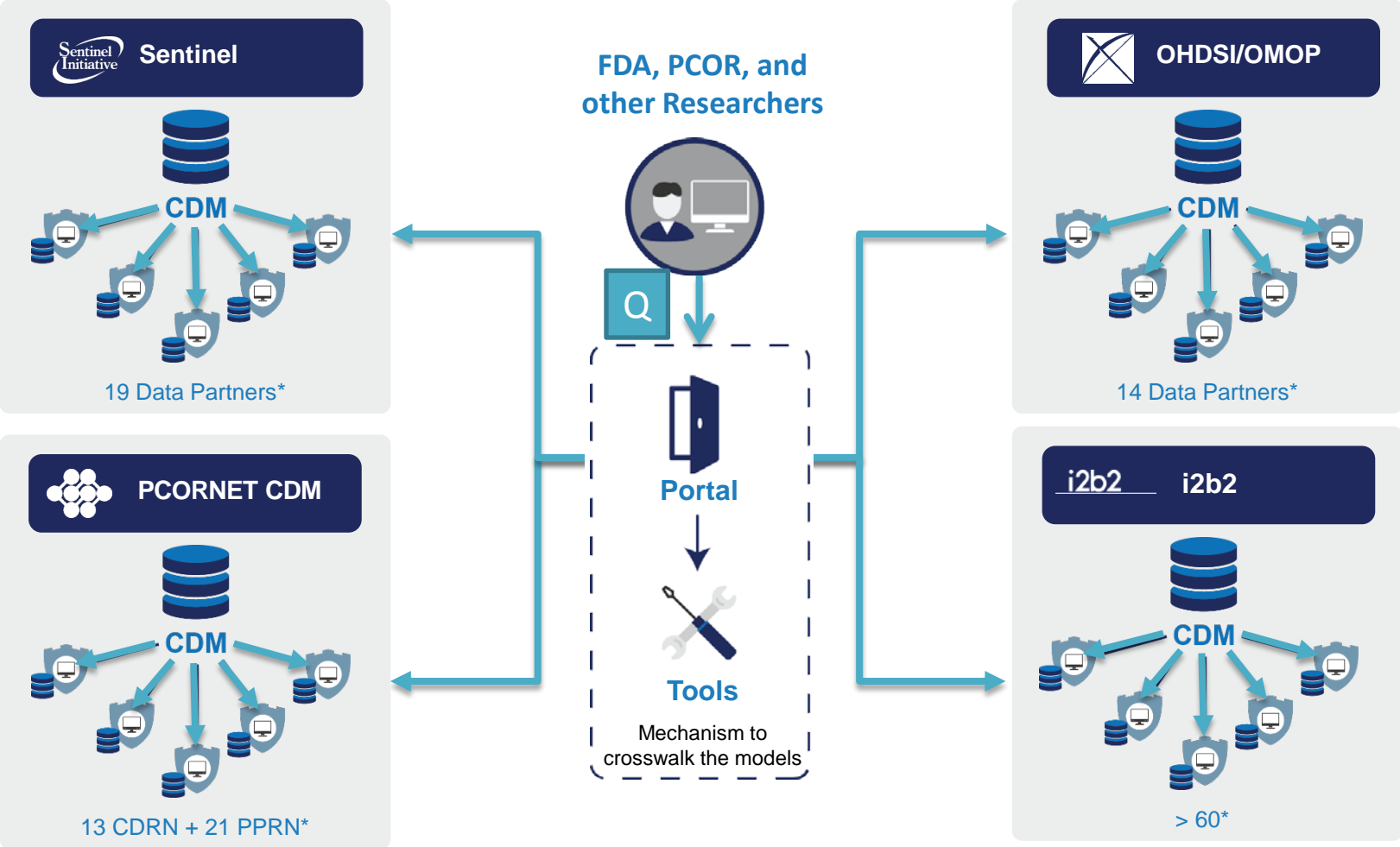
Develop the method to harmonize the Common Data Models of various networks, allowing researchers to simply ask research questions on much larger amounts of Real World Data than currently possible, leveraging open standards and controlled terminologies to advance Patient-Centered Outcomes Research.

# The Concept

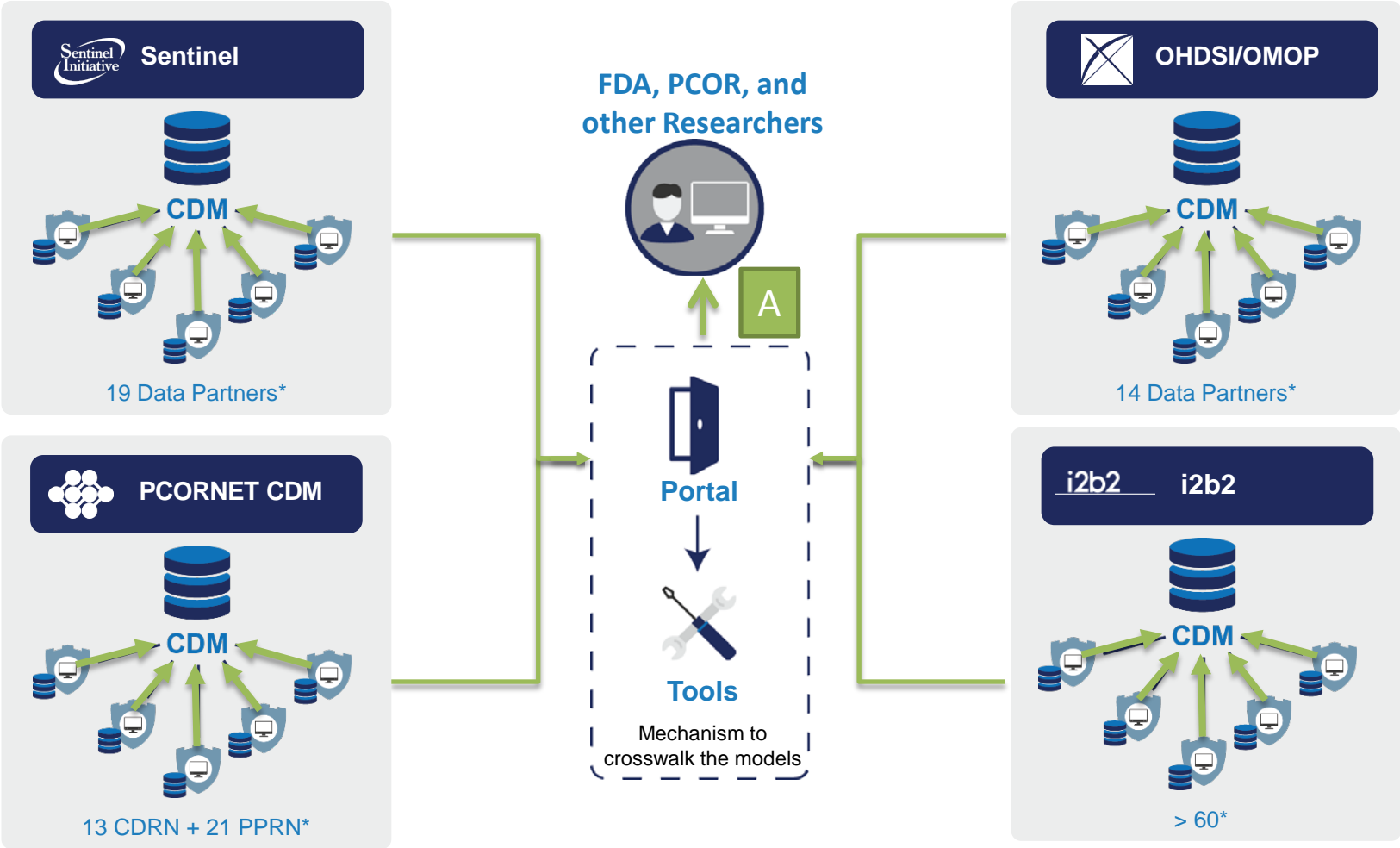


\* See the list of data partners on the back up slides

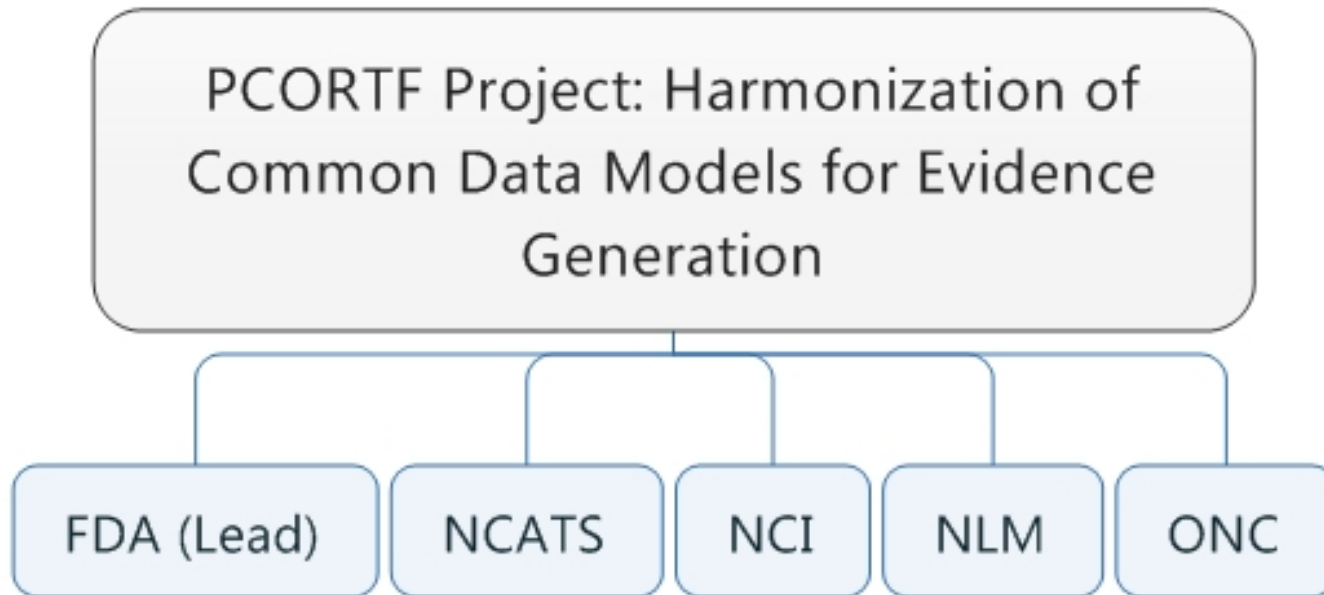
# How Will It Work?



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# Key Players



## Problems to Solve



Networks of observational data use different CDMs.



Open, consensus-based standards might not be leveraged in these CDMs (ex: CDISC, HL7)



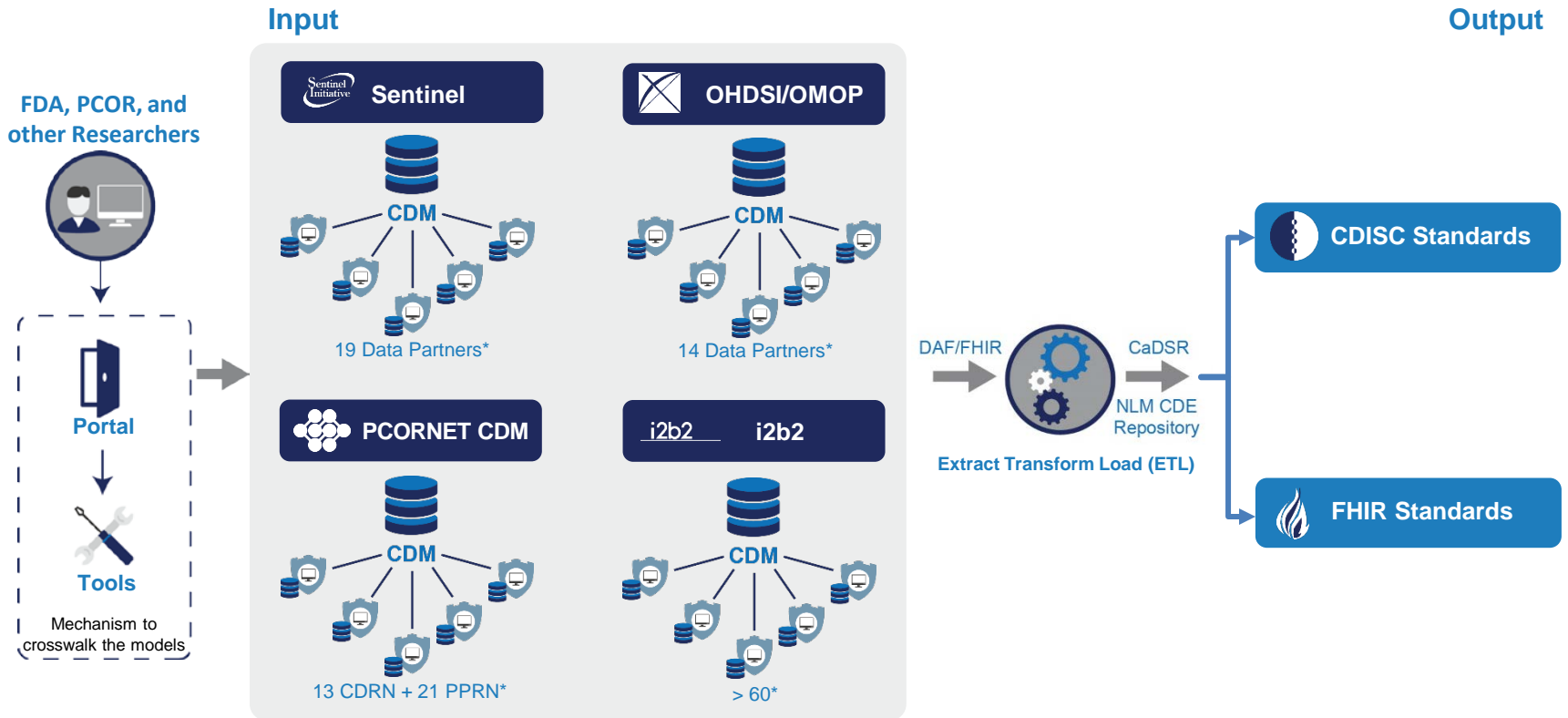
There is a need to facilitate interoperability among these collaborations

# Extended Solution



1. Develop a general framework (i.e., tools, processes, policies, governance and standards) for the transformation of various CDMs, curation, maintenance and sustainability.

## Emergent Data Architecture





# Additional Goals of CDM Harmonization Project



2. Assess the value of the developed CDM harmonization mechanisms by demonstrating research utility for safety evaluation of cancer drugs that use the body's immune system (PD1/PDL1 inhibitors) with a focus on patients with autoimmune disorders.
3. Reuse infrastructure developed by currently-funded OS PCORTF projects (NLM Common Data Elements (CDE) Repository, Data Access Framework (DAF), ....)
4. Leverage open standards and controlled terminologies to Patient-Centered Oriented Research.
5. Test methods and tools developed by the collaborative on the universal CDM mapping and transformation approach.

## Benefits to Public Health and Research

- Evaluates potential safety concerns
- Supports provisions of 21<sup>st</sup> Century Cures Act
- Supports the Patient Centered Outcomes Research goals
- Enhances regulatory decisions by providing reviewers with access to a larger network of RWD data
- Supports the goals outlined in the All of us (Precision Medicine Initiative)