

# Collaborations with Patient Groups: Perspectives from the PARTNERS PPRN and the Childhood Arthritis & Rheumatology Research Alliance (CARRA) Registry

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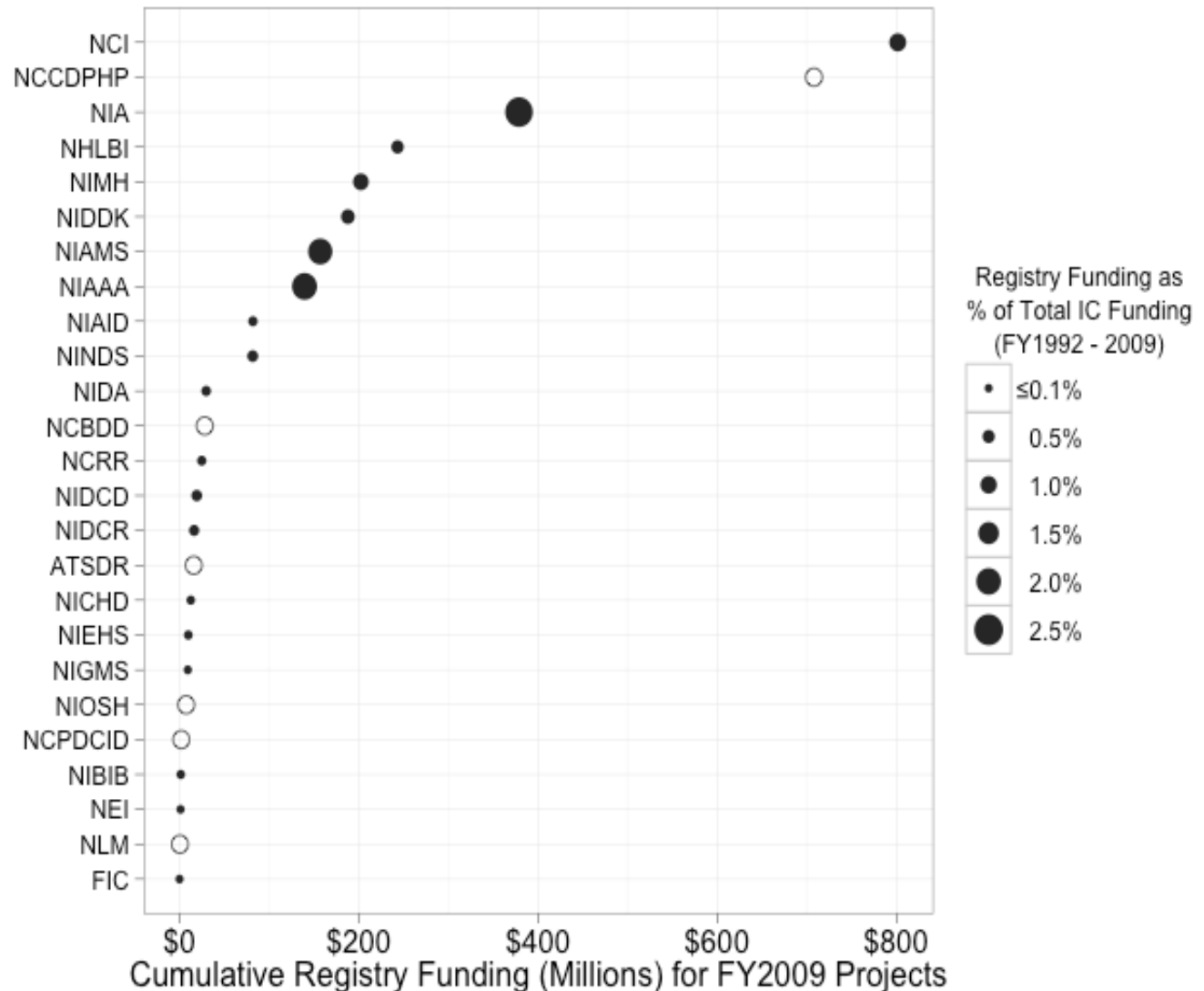
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# \$3.162B NIH & CDC Registry-related Project Funding:

FY 2009 active projects by Institute or Center (cumulative since 1992\*, in millions, FY 2008 CPI-adjusted \$)

**ATSDR:** Agency for Toxic Substances & Disease Registry  
**FIC:** Fogarty International Center  
**NCBBB:** Nat'l Center on Birth Defects & Developmental Disabilities  
**NCCDPDP:** Nat'l Center for Chronic Disease Prevention & Health Promotion  
**NCI:** National Cancer Institute  
**NCPDCID:** Nat'l Center for Preparedness, Detection, & Control of Infectious Diseases  
**NCRR:** Nat'l Center for Research Resources  
**NEI:** National Eye Institute  
**NHLBI:** Nat'l Heart, Lung, & Blood Institute  
**NIA:** National Institute on Aging  
**NIAAA:** Nat'l Inst on Alcohol Abuse & Alcoholism  
**NIAID:** Nat'l Institute of Allergy & Infectious Diseases  
**NIAMS:** Nat'l Institute of Arthritis & Musculoskeletal & Skin Diseases  
**NIBIB:** Nat'l Institute of Biomedical Imaging & Bioengineering  
**NICHD:** Nat'l Institute of Child Health & Human Development  
**NIDA:** Nat'l Institute on Drug Abuse  
**NIDCD:** Nat'l Institute on Deafness & Other Communication Disorders  
**NIDCR:** Nat'l Institute of Dental & Craniofacial Research  
**NIDDK:** Nat'l Institute of Diabetes & Digestive & Kidney Diseases  
**NIEHS:** Nat'l Institute of Environmental Health Sciences  
**NIGMS:** Nat'l Institute of General Medical Sciences  
**NIMH:** Nat'l Institute of Mental Health  
**NINDS:** Nat'l Institute of Neurological Disorders & Stroke  
**NIOSH:** Nat'l Institute for Occupational Safety & Health  
**NLM:** National Library of Medicine



\*NIH grants & contracts: FY 1992-2009; CDC grants & contracts: FY 1995-2009

# Background:

## The Childhood Arthritis & Rheumatology Research Alliance (CARRA) Registry



- CARRA
  - Investigator-led group of essentially every North American pediatric rheumatologist and scientist in the field (N = ~600)
  - Strong and enduring partnerships with patient/parent advocacy groups since establishment in 2002
- CARRA Registry: Investigator-led, patient partnership model
  - ‘Legacy Registry’ est. 2010 (NIH ARRA RC2 ‘Grand Opportunities’ grant) – ~10,000 subjects, encompassing all pedi rheum diseases
  - ‘Rebooted’ 2015 as a 21CFR11-compliant, long-term safety and outcomes, investigator-led, clinical trials network including
    - Phase III and Phase IV FDA clinical trials
    - Consensus treatment plans and pragmatic trials
    - Diverse array of NIH, PCORI, AHRQ, CDC, pharma-sponsored studies

# CARRA Research Portfolio

Randomized Multicenter Clinical Trials

Translational Studies (ancillary to large studies and stand alone)

Comparative  
Effectiveness Research

Pharmacosurveillance  
Program

Biospecimen  
Repository

Observational/Natural History  
Studies

Implementation of Best  
Practices

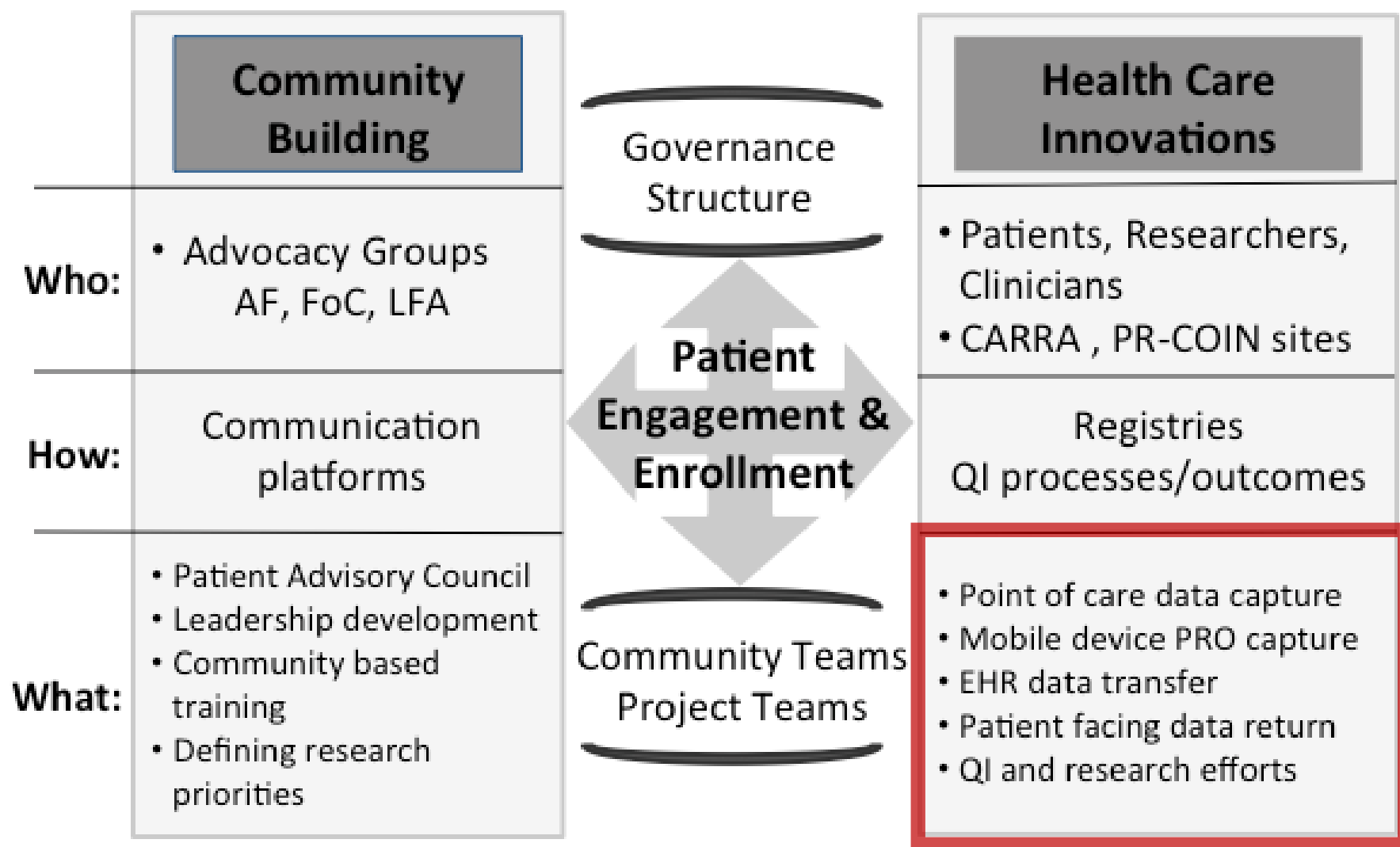
CARRA Registry Data Collection Platform

# ***PARTNERS Patient-Powered Research Network:*** **Patients, Advocates, Research Teams** **Network for Research and Service**

- Established as a PCORnet PPRN in 2014
- Infrastructure fully incorporated into CARRA since 2019



# ***PARTNERS Patient-Powered Research Network:*** Initial vision (2014)



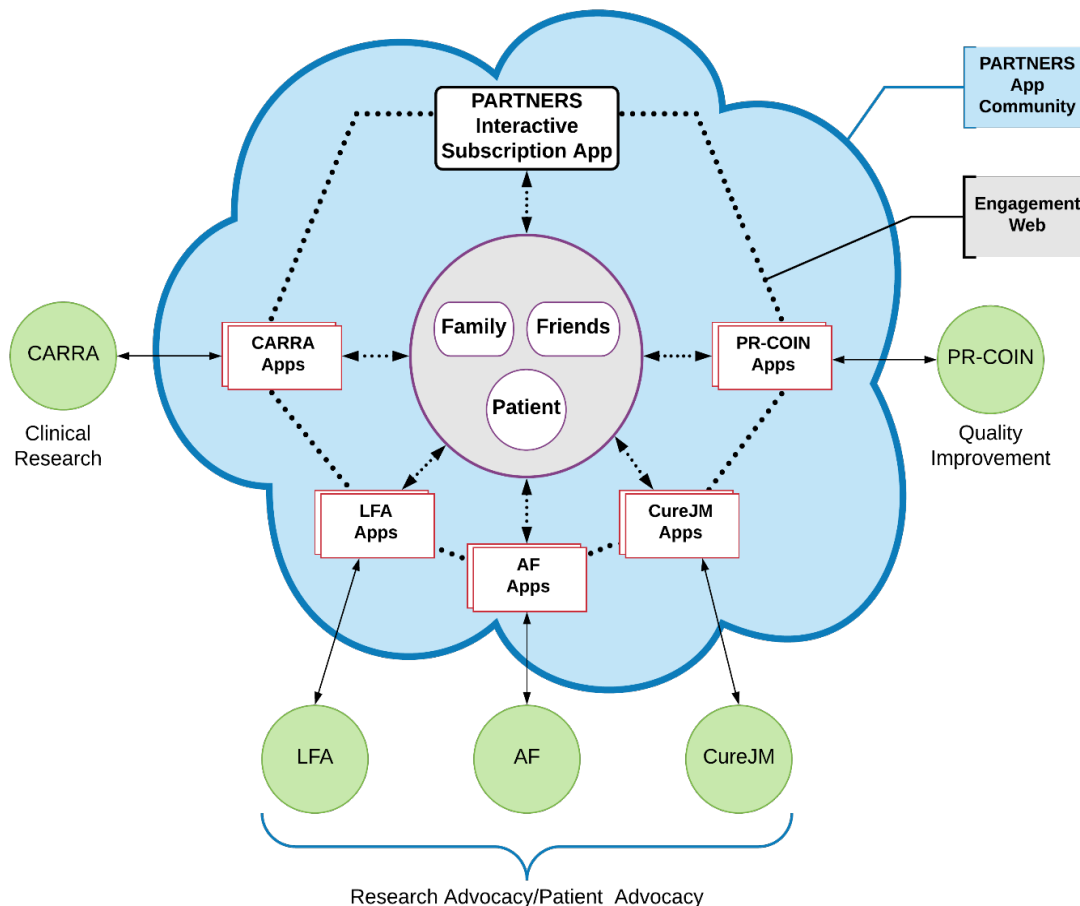
# Examples of CARRA PCOR Research and Data-Centric Activities

- STOP-JIA (PCORI): Observational trial to optimize start-time of biologic therapies in polyarticular Juvenile Idiopathic Arthritis (JIA)
- LIMIT-JIA (PCORI): RCT for use of abatacept to prevent extension of limited JIA
- Web of Engagement (PCORI): Enabling Real-Time, Personalized Engagement in Research: An App-based Approach
- 21CFR11-compliant PRO data exchange standardization (FDA): FHIR Implementation Guide
- PEPR Consortium (NIAMS): Advancing the Science of Pediatric Patient Reported Outcomes for Children with Chronic Disease

# PCOR Lessons Learned

1. Engage (and train) Patient-Partners who have lived experience for the research being done.

PARTNERS 'Web of Engagement'

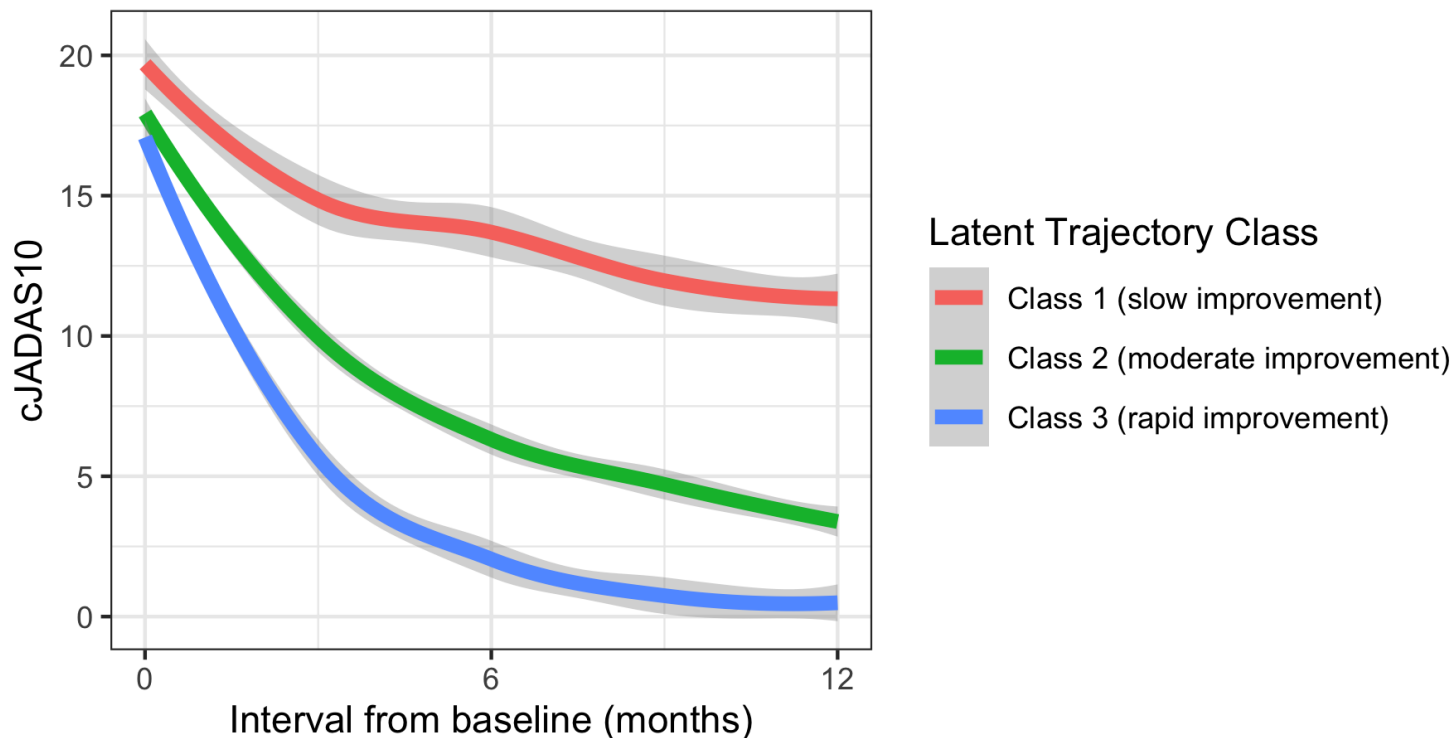


- Each PARTNERS organization provides organization and/or study-specific engagement and research apps (“community apps”)
- The “**Subscription App**” is a separate, gateway app in orchestrates a “Web of Engagement” with users
  - Facilitates navigation of the App Community
  - Enables personalization of notification preferences, receipt of notifications
  - Provides links to curated content



# PCOR Lessons Learned

## 2. Focus on obtaining data for outcomes that matter to patients



# PCOR Lessons Learned

## 3. Leverage use of **standard APIs** to an equal (or greater) degree than data model standardization/harmonization

### FHIR PRO IMPLEMENTATION GUIDE

- WIP and still under development
- Illustrates use of Questionnaire and QuestionnaireResponse resources
  - ...and Adaptive\* versions of these resources for Item Response Theory implementations
- SMART on FHIR Interaction
- Often prescriptive about technology

<http://hl7.org/fhir/us/patient-reported-outcomes/2019May/>

