

# *Innovation in Translational Science*

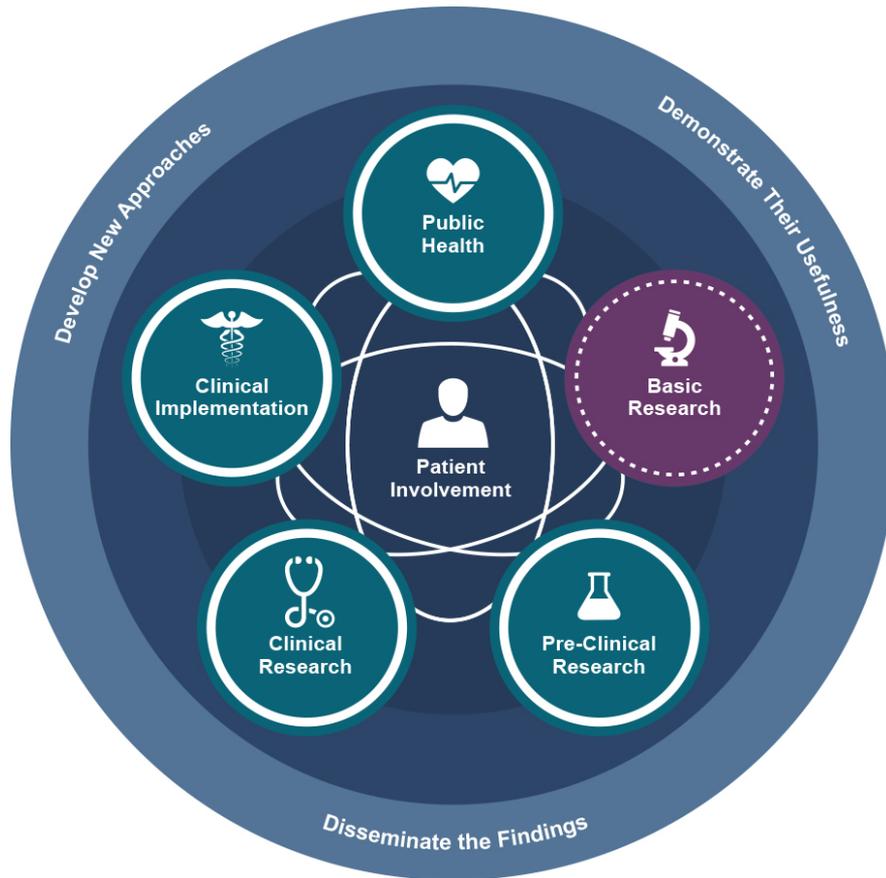
## *Update from the CTSA and ORDR Programs*

**Petra Kaufmann, MD, MSc**  
**Director, Division of Clinical Innovation**  
**Director, Office of Rare Diseases Research**

**NCATS ADVISORY COUNCIL MEETING**  
**JANUARY 14, 2016**

NCATS

# NCATS extramural translational science: accelerating progress for common and rare diseases



- Getting from discovery to health benefits faster
- Focusing on patients and communities as active partners
- Promoting synergy and coordination among stakeholders
- Improving tools and methods
- Training and cultivating the translational science workforce

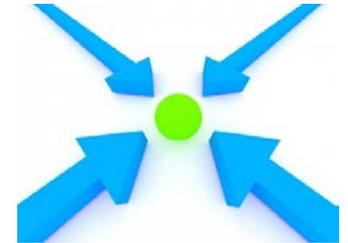
# Streamlining Multisite Clinical Studies

- The problem: current atomized system is inefficient, costly and often ineffective
  - Duplicative IRB reviews among sites
  - Subcontracting harmonization among institutions delays start-up
  - Duplicative investigator/site qualification
- Solutions in progress
  - Centralized IRB review (reliance agreements, IT solutions)
  - Streamlined contracting (pre-negotiated master agreements)
  - GCP training across CTSA sites



# IRBrelly

## One IRB per multisite study



### Accomplishments:

- ✓ IRB Reliance Agreement
- ✓ Workflow
- ✓ IT Solutions
- ✓ Pilot test underway
  - CARRA: Childhood Arthritis & Rheumatology Research Alliance  
Laura Schanberg (Duke University)
  - Feedback and testing to revise and enhance processes

### Team:

- Lead Investigator: Alan Green, Dartmouth
- Senior Reliance Advisor: Barbara Bierer, Harvard
- Regulatory Leads: Sabune Winkler, Harvard; and Nichelle Cobb, University of Wisconsin
- Informatics Lead: Amarendra Das, Dartmouth
- Critical contributions and support: CTSA Program investigators and their teams

Coordinating with: PCORnet, CTTI, RDCRN and others

# Innovation for Multisite Studies: Streamlined Contracting

Accelerated Confidential Disclosure  
Agreement  
**ACDA**

Accelerated Clinical Trial Agreement  
**ACTA**

Accelerated Subcontracting

<https://www.ara4us.org/>

Khosla, Bernard, Salberg, Bruce, Edwards et al, and CTSA teams

# Harmonizing training across the CTSA Network

## Translational Competencies:

- Agreement on Good Clinical Practice (GCP) standards
- Translational research competencies
- Shared curriculum and training opportunities, including for
  - first-time PIs
  - Clinical research coordinators
  - Clinical research personnel
  - Community members
- Streamlined training and site qualification
- Less redundancy
- Increased opportunities to use online platforms

# Improving Participant Recruitment Accrual to Clinical Trials (ACT)

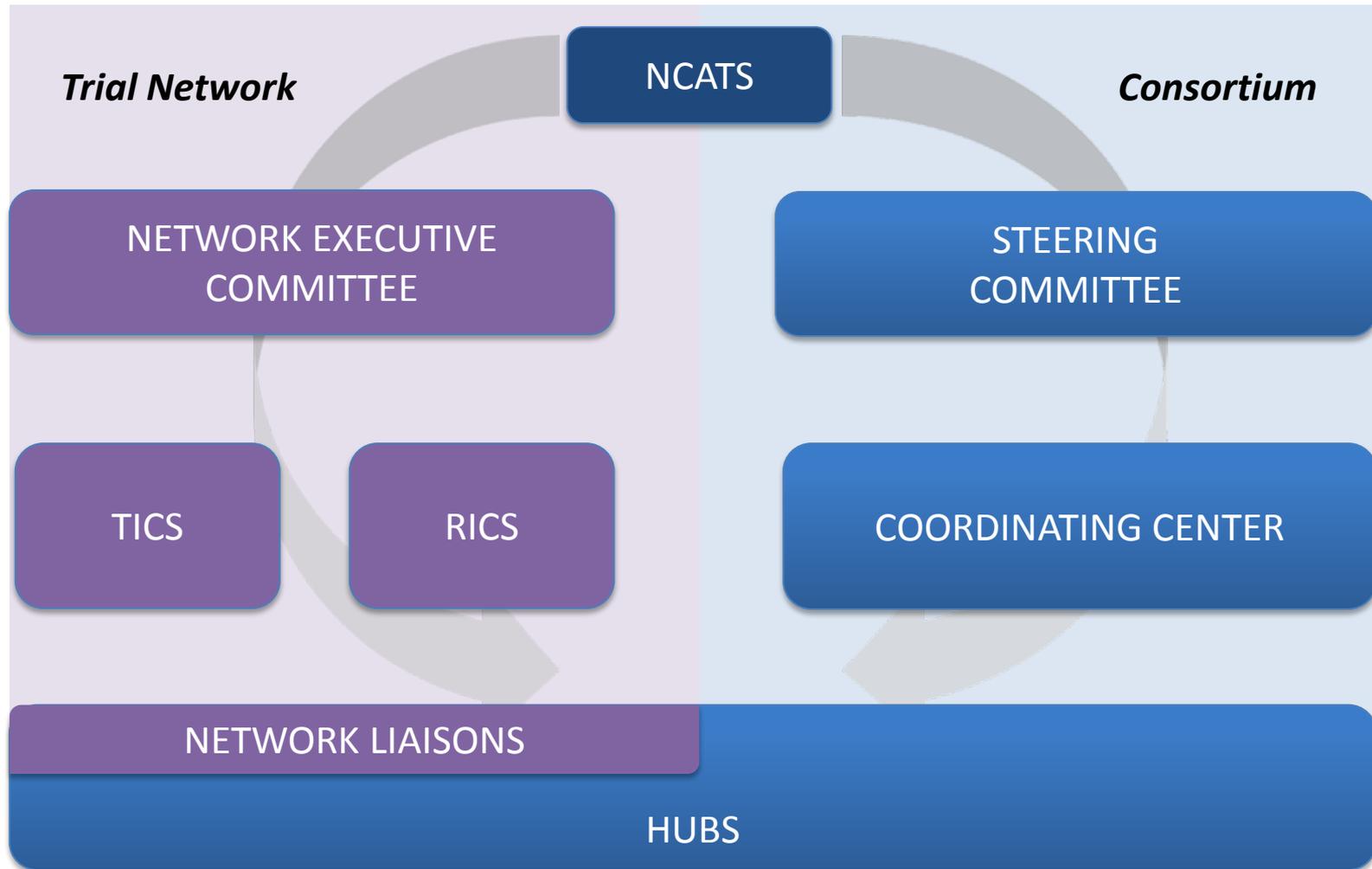


- ✓ Technical implementation at 22 sites
- ✓ Infrastructure and SOPs for governance, technology and regulatory issues for identification of study participant cohort across multiple sites, including IRB approval from all participating sites
- ✓ ACT ontology: Demographics, Diagnoses, Procedures, Visit Details, Medications, Laboratory Test Results
- ✓ Pilot queries across more than 20 sites:
  - Rheumatoid arthritis, Hepatitis, TACT2
- ✓ Leveraging synergy with PCORnet
  - PCORnet-CTSA iDTF meeting in San Francisco, monthly calls
- ✓ Promoting standards, shared terminologies and computable phenotypes

Steven Reis, Lee Nadler, Robert Toto, Gary Firestein, et al.



# CTSA Program



# RFA-TR-15-002 - CTSA Network Trial Innovation Centers (TICs)

- Centers of excellence that facilitate and innovate the implementation of multi-site clinical studies by the CTSA Network
- Activity code: U24
- Key dates:
  - Released: June 5, 2015
  - Application Due: September 15, 2015
  - Scientific Merit Review: February 2016
  - Advisory Council Review: May 2016
  - Earliest Start Date: June 2016
- Award
  - Up to \$4M (total cost per center) to fund 3 awards in 2016
  - Up to 7 years
- Partner: National Institute on Aging (NIA)

# RFA-TR-15-042 - CTSA Network Recruitment Innovation Centers (RICs)

- Activity code: U24
- Key Dates:
  - Applications received: July 22, 2015
  - Scientific Merit Review: November 2015
  - Advisory Council Review: January 2016
  - Earliest Start Date: February 2016
- Award
  - Up to \$3M (total cost per center and year) to fund 2 awards in 2016
  - Up to 5 years
- Partner: National Library of Medicine (NLM)

# Fostering collaboration to transform translational science

- Collaborative Innovation Awards

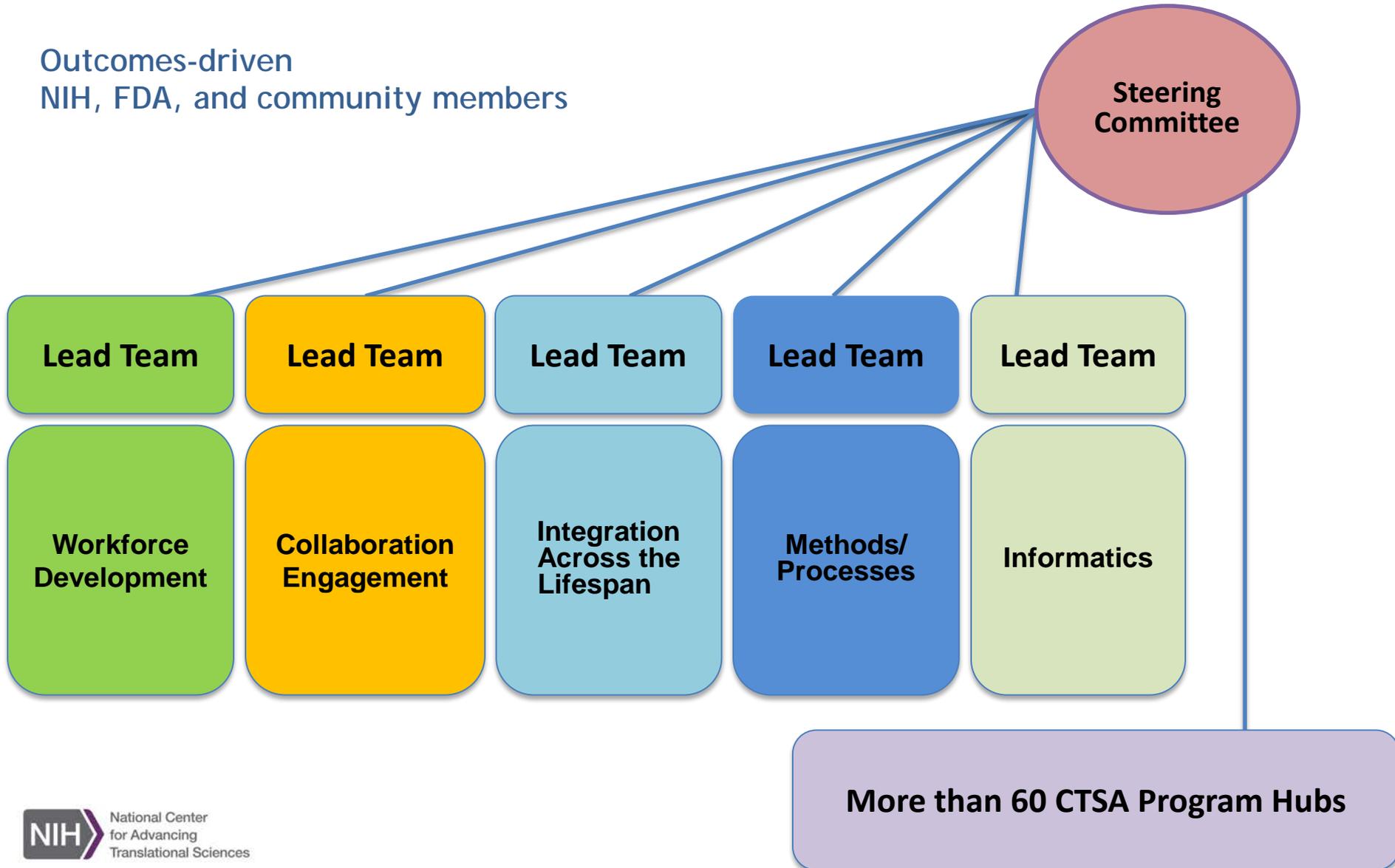
PAR-15-173 (X02) / PAR-15-172 (U01)

- Develop new technology, method or approach to address roadblock in translational science
- Involve collaboration at least 3 CTSA Program hubs
- Vigorous response, many X02 submissions
- Identified exciting applications for U01 submission spanning broad spectrum of translational science including:
  - Training
  - Informatics
  - Community engagement
  - Biomedical approaches

First U01 deadline: February 2016

# Streamlined CTSA Program Communications Structure: Domain Task Forces

- Outcomes-driven
- NIH, FDA, and community members



# Common Metrics

- Data for the strategic management of the CTSA Program
  - Local and national
  - Focus on impact
  - Not evaluation but management tool
- Process is bottom-up with active engagement of PIs, evaluators, administrators, coordinators and others
- First set of templates adopted at December PI meeting
- Now in the process of developing SOPs and launching pilot study

# PIs Who Have Worked on the Common Metrics



# Evaluator Workgroup Leads

## Collective Impact at Hubs

Arthur Blank

*Albert Einstein  
College of  
Medicine*



## Workforce Development

Doris Rubio

*University of  
Pittsburgh*

## Consortium 2.0

Julie Rainwater

*University of  
California, Davis*



## Hub Resources and Services

Bill Trochim

*Weill Cornell  
Medical College*

**NCATS Metrics Team:** Meryl Sufian, Samantha Jonson, Redonna Chandler, Abby Bronson and DCI Program Directors

**Tufts Metrics Team:** Harry Selker, Pieta Blakely, Denise Daudelin, Lisa Welch, Debra Lerner

**Results-based Accountability:** Phil Lee

# We thank the workgroup evaluators:

## Collective Impact at Hubs

- **Pamela Davidson (UCLA) Co-lead**
- **Boris Volkov (U. Minnesota) (co-lead)**
- Beatrice Boateng (U. Arkansas, Medical Sciences)
- Beth Tigges (UNM)
- Dagobert Soergel (U. at Buffalo)
- Gaurav Dave (UNC Chapel Hill)
- Harold Pincus (Columbia U. Medical Center)
- Jillian Harvey (Medical U. of S.C.)
- Kristi Holmes (Northwestern)
- Laura Weisel (Harvard)
- Leonard Bickman (Vanderbilt)
- Millie Harris (OSU)
- Raul Caetano (UT Southwestern)
- Tamara Havermahl (U. Michigan)
- Tesheia Johnson (Yale)

## Consortium 2.0

- **Melanie Funes (USC) (Co-lead)**
- **Lisa Welch (Tufts) (Co-lead)**
- Becky Moen (Duke)
- Deborah Fournier (Boston U.)

- Gregory Tong (UCSF)
- Helen Parsons (UT Health Science Center at San Antonio)
- John Nash (U. Kentucky)
- Lisa Jackson (Stanford)
- Margaret Schneider (UC Irvine)
- Michelle Miller (The Scripps Research Institute)

## Hub Resources and Services

- **Janice Hogle (UW Madison) (Co-lead)**
- **Joe Hunt (Indiana U.) (co-lead)**
- Adrienne Zell (Oregon Health and Science U.)
- Christine Weston (Johns Hopkins)
- Deborah Chavis-Keeling (NYU School of Medicine)
- Eric Nehl (Emory)
- Knut M. Wittkowski (The Rockefeller U.)
- Laura Meiners (Mayo Clinic)
- Lisle Hites (UA Birmingham)
- Meridith Mueller (Medical College of Wisconsin)
- Michael Quinn (U. Chicago)
- Negin Fouladi (U. Texas)

- Priyanka Nasa (UI Chicago)
- Rachel Boren (U. Washington)
- Robert Morgan (UT Health School of Public Health)
- Rosalina Das (U. Miami)
- Tanha Patel (Wake Forest U.)
- Veronica Thomas (Howard U.)

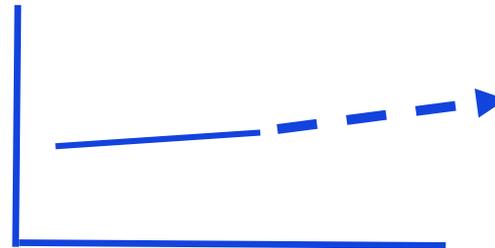
## Workforce Development

- **Clara Pelfrey (Case Western) (Co-lead)**
- Ann Dozier (U. Rochester)
- Cecilia Patino-Sutton (U. Southern California)
- Debora DiazGranados (Virginia Commonwealth U.)
- Elizabeth Palombo (Washington U. St. Louis)
- Jodi Cullum (U. Utah)
- John T. Farrar (U. Penn)
- Karen McDonnell (George Wash. U.)
- Kevin Wooten (UT Medical Branch)
- Kimberly G. Stevenson (Boston U.)
- Linda Scholl (UW Madison)
- Patrick Barlow (U. Iowa)

# The CTSA Common Metrics: A Tool for Strategic Management and Collaboration

How are we doing?

Track data about impact



Why?

Rigorously analyze the underlying factors

*Partners?*

*Engage partners who can help*

*Options?*

*Innovate strategies*

Actions?

Implement the most promising strategies

# Office of Rare Diseases Research

## Rare Diseases Clinical Research Network (RDCRN) Program

- ❖ 22 consortia at 250 institutions worldwide
- ❖ Studying 282 rare diseases with more than 90 active protocols
- ❖ More than 130 patient advocacy groups participating

## Genetic and Rare Diseases (GARD) Information Center Program

- ❖ Provides up-to-date information to patients, families, researchers, and the public about rare or genetic diseases.
- ❖ Information provided by information specialists and on the NCATS/GARD website <https://rarediseases.info.nih.gov>

## NCATS Scientific Conferences Program

- ❖ ORDR manages a committee to Identify scientific opportunities for rare and common diseases and evaluate applications

## Global Rare Diseases Patient Registry Data Repository/GRDR® Program

- ❖ Will provides a “one-stop shop” for rare diseases data from registries in the USA and elsewhere
- ❖ Will provide ability to conduct cross-disease analysis and recruitment

# NCATS Toolkit Project

- Bringing together patient advocacy groups (PAGs)
- Surveying landscape of translational research tools and resources
- Providing online roadmap
- Identifying gaps
- Providing training and information for PAGs
- Coordinating with CPAG
- Building on existing strength and partnerships with community

**Program design group:**

Eckstein, Wilson, Brown,  
Kennedy, Bartek

# Save the Date: Rare Disease Day at NIH

## Feb. 29, 2016

- National Institutes of Health Main Campus, Bethesda, Maryland, 8:30 a.m. – 3:30 p.m.
- Features presentations, posters, exhibits, tours
- Registration and event information at:  
[ncats.nih.gov/rdd](http://ncats.nih.gov/rdd)



# Summary

- Successful demonstration projects
  - IRB reliance
  - Using EHR for research participant recruitment
- Network resources on track for launch in 2016
  - Trial Innovation Centers
  - Recruitment Innovation Centers
- Collaborations underway
  - Communication via Domain Task Forces
  - Innovative Collaborations Award
- Metrics initiative met first milestones
- ORDR partnering with patient groups to create toolkit and training resources

# Questions