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
IRBrely
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: Amarenda 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.
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

More than 60 CTSA Program Hubs
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

**Consortium 2.0**
- Julie Rainwater
- University of California, Davis

**Workforce Development**
- Doris Rubio
- University of Pittsburgh

**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)

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.)
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- 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)

Consortium 2.0
- Melanie Funes (USC) (Co-lead)
- Lisa Welch (Tufts) (Co-lead)
- Becky Moen (Duke)
- Deborah Fournier (Boston U.)
- 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
  - Engage partners who can help
  - 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 provide 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
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