How can the CTSAs support Rare Disease Research?

Symposium: Best Practices in Clinical Study Design for Rare Diseases, Washington, DC 30 April 2013

MARY PURUCKER, MD, PHD
MEDICAL OFFICER/PROGRAM OFFICIAL
NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCE (NCATS), NIH
6701 DEMOCRACY BLVD
BETHESDA, MD 20892

Barry J. Byrne, M.D., Ph.D.
Professor and Associate Chair of Pediatrics,
Molecular Genetics & Microbiology
Director, Powell Gene Therapy Center
University of Florida, School of Medicine
Gainesville, Florida 32610-0266

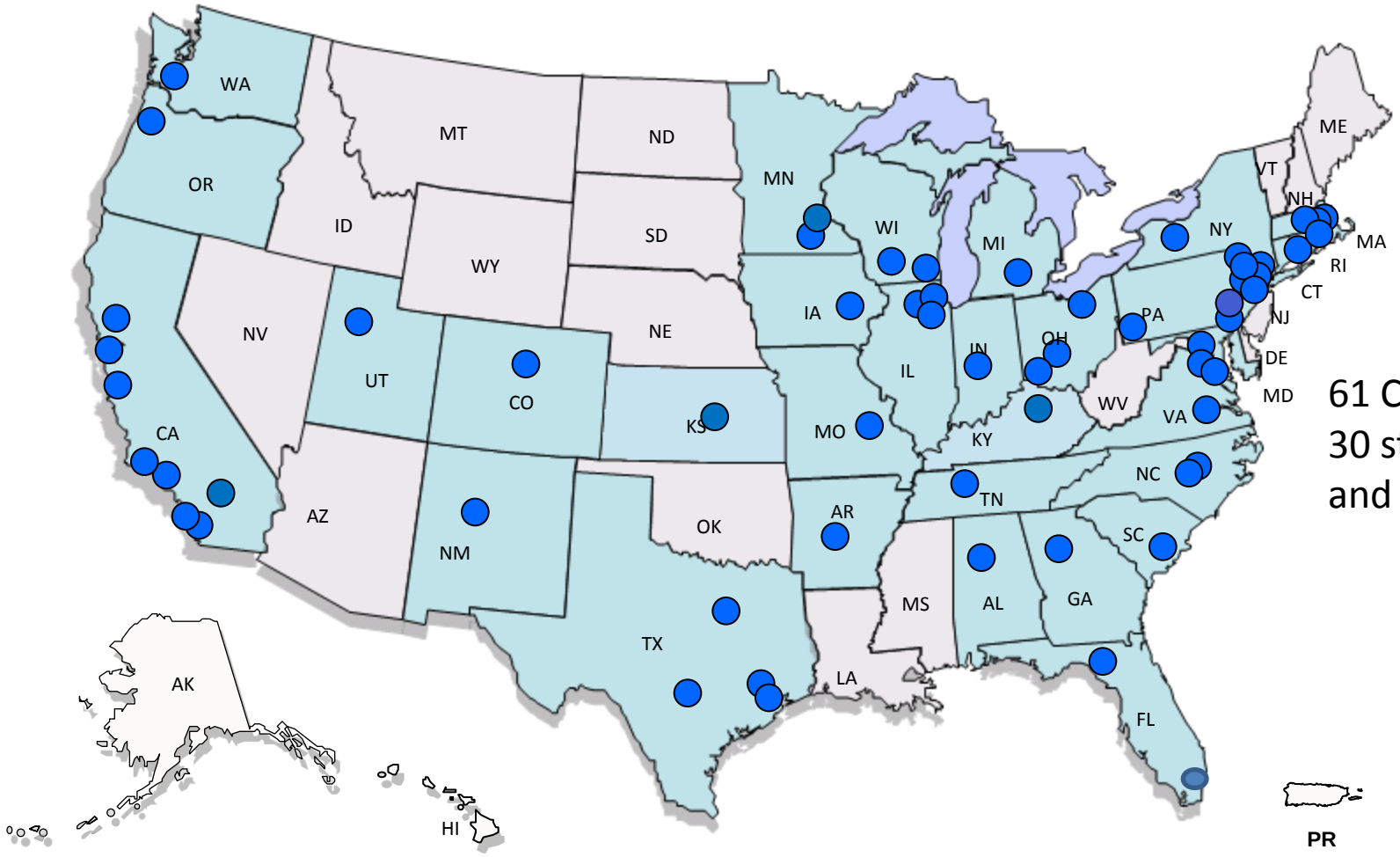
NCATS

NIH National Center for Advancing Translational Sciences

CTSA Clinical & Translational Science Awards

Accelerating Discoveries Toward Better Health

Visit CTSAcentral.org to learn more.



61 CTSAs in 30 states and DC

● = CTSA Sites

□ = CTSA States

NIH National Center for Advancing Translational Sciences

CTSA Consortium Steering Committee

- Governance body of the CTSA Consortium comprised of PIs from all CTSA sites
- Annual Meeting held October 10-11, 2013
- CTSA support for rare disease research and for undiagnosed disease program were the subject of a breakout session
- Many areas of synergy, rare disease research considered a “natural fit”
- Potential for approaches that lead to cost efficiency for diagnosis, patient care, and long-term follow-up

CTSAs: Resources for Study Design & Product Development

- CTSA have multiple components that were required under the original RFA: clinical research facilities with trained personnel, biostatistics, research design, regulatory support, ethics, bioinformatics, research education, community engaged research, and commercialization resources
- Some CTSAs have specialized cores: genetics, genomics, biorepositories, Phase I units, drug discovery cores (± HTS, pharmaceutical libraries), medical device prototyping
- Consortium-wide resources available: www.ResearchMatch.org web-based patient recruitment portal, REDCap data management tool, IRBShare, research networking tools such as VIVO or Eagle-i

CTSA Rare Disease Workgroup

- Administratively linked to CTSA Consortium Child Health Oversight Committee (CC-CHOC)
  - Consortium leadership committee (with CCSC/Steering Committee)
- Co-chaired by Barry Byrne (UFL), Peter Merkel (UPenn), and Berch Griggs (U Rochester)
  - Includes investigators with pediatric and adult research interests
  - Strong ties to the RDCRN
  - Oct 2012 co-sponsorship of Conference on Clinical Research on Rare Diseases
- Example of shared collaborative effort: Pediatric Point Person Project

Point Person Project:
Finding Collaborators/Initiating Multisite Studies

- Idea first proposed by the CTSA RDWG as a means to identify rare disease collaborators at CTSA sites across the Consortium
- Proposal to use VIVO research networking tool implemented across CTSA sites and supported by UFL (led by Mike Conlon)
  - Effort is ongoing
- Concurrent effort by CC-CHOC using volunteers from CTSA sites was launched last year

Point Person Project:
Finding Collaborators/Initiating Multisite Studies

- Concept: A central contact for a CTSA site can be used by individual investigators or industry to recruit experts for protocol development or implementation
- "Point Person" is designated at each CTSA site to review and respond to collaborative opportunities
- Point Person functions as a navigator – direct trial opportunities to local investigators with appropriate expertise and potential interest

Protocol Synopsis

- Potential sponsors submit a protocol summary to CTSA Consortium Coordinating Center (C4)
- Title
- Study population
- Rationale (drug/device may be de-identified)
- Study objectives (including any regulatory requirements for the study)
- Inclusion/exclusion criteria
- Overview study design
- Number of patients to be enrolled - how long will the study run

Process Flow

- Coordinating Center logs in study, sends to CC-CHOC coordinator(s) for approval, then distributes to 86 contacts from 55 CTSA sites
- Hyperlink at the bottom of the synopsis form leads to a response form
- Clicking on “interested” or “not interested” box brings separate drop down menus
- List of interested parties are forwarded to the Sponsor within 72 hours
- Weekly teleconferences with Sponsors to answer questions, provide more information

Progress Report

- 23 protocols evaluated and distributed in the 1st year
  - Source:
    - 15 CRO
    - 6 Industry
    - 2 Individual Investigators
  - Disease Focus
    - Wide range of disorders affecting children, including rare diseases
  - Age Range
    - Newborn to Adolescent (most ages 6-17)
- After a review of the full protocol, 50% of interested investigators decided to participate

Lessons Learned

PPP temporarily halted to refine model and develop metrics

- Project management and data handling/tracking crucial to success
  - Resource intensive and not sustainable
  - Cannot be supported indefinitely by C4
- "Next steps" and outcome tracking
  - There is no active tracking of the project once the investigators are matched or the investigators are matched with the sponsor
  - Downstream information gathering is dependent upon unstructured and voluntary reporting by investigators or sponsor

Issues for Discussion

Is PPP a suitable model for Rare Disease Research?

- Expanded use of VIVO might enhance or streamline the search for collaborators.
  - Augment the Point Person at CTSA site
- Possible to combine with resource discovery tool such as Eagle-I
  - Research services, core laboratories, instruments
- Project management would still be needed - Who would provide?
  - Trusted and ideally independent of sponsor and investigators
  - How to fund?
- Need for “enhanced” data management such as a DCC
  - Service provided by C4 not equivalent to DCC support