

Rare Diseases Clinical Research Network (RDCRN)

- A MODEL FOR COLLABORATIVE RARE DISEASES RESEARCH WITH PATIENT ADVOCACY GROUPS AS PARTNERS

NCATS

RDCRN: Background Information

- Established in 2003 and expanded in 2009
- 3rd cycle renewal in 2014:
 - a Network of 22 distinct multi-site Clinical Research Consortia (U54 - no more than \$1.25M Total Cost/year)
 - Each Consortium studies at least three related rare diseases
 - A central Data Management and Coordinating Center (DMCC)
 - Involves Patient Advocacy Groups - CPAG
 - 10 NIH ICs as research partners

About RDCRN

- Each consortium conducts at least two multi-site clinical studies, has a training program, and pilot project program.
- Collectively, the RDCRN is studying 200 rare diseases in natural history and clinical trials at 240 clinical sites located in the US and in 14 countries.
- There are more than 90 active protocols.
- 29,000 patients have enrolled in clinical studies.
- There have been 174 trainees.
- There are 2,290 collaborative consortium members.
- There are 98 PAGs as research partners.

<http://rarediseasesnetwork.epi.usf.edu/>

NIH

NCATS, NCI, NHLBI, NIAID,
NIAMS, NICHD, NIDCR, NIDDK,
NIMH, NINDS, ODS

Dystonia
Coalition

Coalition of Patient
Advocacy Groups
(CPAG for RDCRN)

Porphyria Rare Disease Clinical
Research Consortium

North America Mitochondrial
Diseases Consortium

Primary Immune Deficiency
Treatment Consortium

Brittle Bone Disorders
Consortium

Chronic Graft Versus
Host Disease

The Data Management and
Coordinating Center

Urea Cycle Disorders
Consortium

Brain Vascular
Malformation Consortium

Genetic Disorders of
Mucociliary Clearance

Consortium of Eosinophilic
Gastrointestinal Disease Researchers

Rett, MECP2 Duplications
and Rett-Related
Disorders Consortium

Sterol and Isoprenoid
Diseases Consortium

Autonomic Disorders
Consortium

Developmental Synaptopathies
Associated with TSC, PTEN
And SHANK3 Mutations

The Frontotemporal Lobar
Degeneration Clinical
Research Consortium

Inherited Neuropathies
Consortium

Nephrotic Syndrome
Study Network

Rare Lung Diseases
Consortium

Lysosomal
Disease Network

Rare Kidney
Stone Consortium

Vasculitis Clinical
Research Consortium

Clinical Research in ALS & Related
Disorders for Therapeutic Development



- Collaborative Clinical Research
- Centralized Data Coordination and Technology Development
- Public Resources and Education
- Training