



U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES
National Institutes of Health
ORD, NCRR, NIAMS, NICHD, NHLBI, NIDDK, NINDS,

Coalition of Patient Advocacy Groups (CPAG)

The Data Technology Coordinating Center

Dr. Krischer

**Bone Marrow Failure
Disease Consortium**

Dr. Maciejewski

Rare Lung

Disease Consortium – Dr. Trapnell

UREA CYCLE DISORDERS CONSORTIUM

Dr. Batshaw

Rare Genetic Steroid

Disorders Consortium – Dr. New

Genetic Diseases of

Mucociliary Clearance Consortium – Dr. Knowles

**VASCULITIS
CLINICAL
RESEARCH
CONSORTIUM**

Dr. Merkel

Cholestatic Liver
Disease Consortium
CLiC –Dr. Sokol

CINCH
Consortium for
Clinical Investigations of
Neurological Channelopathies

Dr. Griggs

Rare Thrombotic
Diseases
Consortium – Dr.
Ortel

Angelman, Rett and

Prader-Willi Syndromes Consortium – Dr. Beaudet

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

Purposes of The Cooperative Rare Diseases Clinical Research Network

<http://www.rarediseasesnetwork.org/>

Facilitate clinical research in rare diseases to support :

- **collaborative clinical research in rare diseases, including longitudinal studies of individuals with rare diseases, clinical studies, phase one and two trials, and/or pilot and demonstration projects.**
- **a test bed for distributed clinical data management that incorporates novel approaches and technologies for data management, data mining, and data sharing across rare diseases, data types, and platforms.**
- **promote training of new clinical investigators in rare diseases**

Data and Technology Coordinating Center

- ♦ **Collaboration in design of clinical protocols, data management and analysis**
- ♦ **Develop a coordinated clinical data management system for the collection, storage and analysis of data from multiple diseases and multiple clinical sites**
- ♦ **Develop tools for web based recruitment and referral, cross disease data mining**
- ♦ **Construct a portal for access and integration of public data resources**
- ♦ **Promote communication and coordination of Network (including internet video conferencing, centralized secure website)**

Coordinated Efforts for Successful Orphan Product Development/Rare Diseases Research

- **Industry (Domestic and International, Large and Small)**
- **Academic and Research Community**
- **Multidisciplinary Research Efforts**
- **Medical Specialty Societies**
- **Patient Advocacy Groups**
- **Federal Government**
 - **Research**
 - **Intramural Research Program**
 - **Extramural Research Program**
 - **Regulatory and Research**
 - **Reimbursement**

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