The Rare Diseases Clinical Research Network (RDCRN): Registries and Longitudinal Studies

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Established by the Office of Rare Diseases Research

2003--10 Consortia supported by ORDR, NCRR, NINDS, NIAMS, NICHD, NHLBI, NIDDK

2009--19 Consortia supported by ORDR, NINDS, NIAMS, NICHD, NHLBI, NIDDK, NIAID, NIDCR

167 institutions world-wide

Collectively studying >200 diseases

5842 patients enrolled in 39 studies
**DHHS-NIH**
ORDR, NINDS, NIAMS, NICHD, NHLBI, NIDDK, NIDCR, NIAID

**Lysosomal Disease Network**

**Dystonia Coalition**

**BVMC**

**The Porphyrias Consortium**

**Rare Kidney Stone Consortium**

**Neptune**

**Coalition of Patient Advocacy Groups (CPAG)**

**Inherited Neuropathies Consortium**

**Primary Immune Deficiency Treatment Consortium**

**Angelman, Rett & Prader-Willi Syndromes Consortium**

**NAMDC**

**NAMDC (National Academy of Mucociliary Disease Center)**

**The Data Management and Coordinating Center**

**Genetic Diseases of Mucociliary Clearance Consortium**

**CINCH**

**Consortium for Clinical Investigations of Neurological Channelopathies**

**Clinical Research Consortium for Spinocerebellar Ataxias**

**Salivary Gland Carcinoma Consortium**

**Autonomic Disorders Consortium**

- Collaborative Clinical Research
- Public Resources and Education
- Centralized Data Coordination and Technology Development
- Training
Goals of the RDCRN

• Facilitate clinical research by:
  – Creation of disease focused Consortia
  – Cost-saving research infrastructures
  – Establishing uniform protocols for data collection
  – Making meaningful large-scale studies possible
    • Longitudinal cohorts, pilot projects, and randomized trials

• Directly engage patients and their advocates

• Train new investigators in rare diseases research
RDCRN Study Types

- Genotype – phenotype studies
- Longitudinal / natural history studies
- Biomarker identification
- Clinical trials
- Tissue banks/ bio-repositories
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Longitudinal Study Characteristics

• Physician Enrolled with informed consent.
• Hypothesis Driven.
• Usual care augmented by directed tests and sample collection.
• Common goals:
  – Describe phenotypic variation.
  – Uniform outcome assessments.
  – Identify population for interventional studies.
Contact Registry’s Global Reach

10-15% of the Contact Registry Have non US, non Canada addresses
RDCRN Patient Contact Registry

- Enrollment open to patients with diseases under study by Consortia
- Provides international on-line system for communication, recruitment, research
- 128 diseases represented
- 75544 Registrations
Contact Registry Characteristics

- Patient/family Enrolled with online informed consent.
- Limited data collection; includes PHI.
- Connect patients with providers.
- Common goals:
  - Provide information about RDCRN.
  - Provide information about studies.
  - Identify populations for interventional studies.
  - Vehicle for epidemiological studies.
Automated Communications

• Typical automations:
  – Welcome
  – New Study
  – New Clinical Site
  – Periodic (e.g., every 6 months)

• Automations can be customized by study, disease, or consortium
An automated communication system in a contact registry for persons with rare diseases: Scalable tools for identifying and recruiting clinical research participants

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ABSTRACT

Objectives: Strategies for study recruitment are useful in clinical research network settings. We describe a registry of individuals who have self-identified with one of a multiplicity of rare diseases, and who express a willingness to be contacted regarding possible enrollment in clinical research studies. We evaluate this registry and supporting tools in terms of registry enrollment and impact on participation rates in advertised clinical research studies.

Methods: A web-based automated system generates periodic and customized communications to notify registrants of relevant studies in the NIH Rare Diseases Clinical Research Network (RDCRN). The system provides tools for the registry administrator to enroll and track participants.
RDCRN Patient Contact Registry
Success Stories

• VCRC Reproductive Health Study: Using the Contact Registry, in less than 10 days of distributing study information to Contact Registry participants, the group has met over 10% of its 2000 participant accrual goal.

• BVMC Study of “Modifier Genes in Cerebral Cavernous Malformations”: Contact Registry helped assist site in recruiting participants from outside of the typical geographical region of families accrued into the study.
Thank you!
Technology

- Application service provider
- .Net / Oracle