The Rare Diseases Clinical Research Network (RDCRN) Program: *A Model for Collaboration with PAGs* & Research Resources/Initiatives at NCATS, NIH

RARE DISEASES SYMPOSIUM
RE(ACT) SWITZERLAND: WE CARE FOR RARE
GENEVA, NOVEMBER 10TH, 2016

RASHMI GOPAL-SRIVASTAVA, PHD
DIRECTOR, EXTRAMURAL RESEARCH PROGRAM
(PROGRAM DIRECTOR, RDCRN)
OFFICE OF RARE DISEASES RESEARCH (ORDR) , USA

NCATS

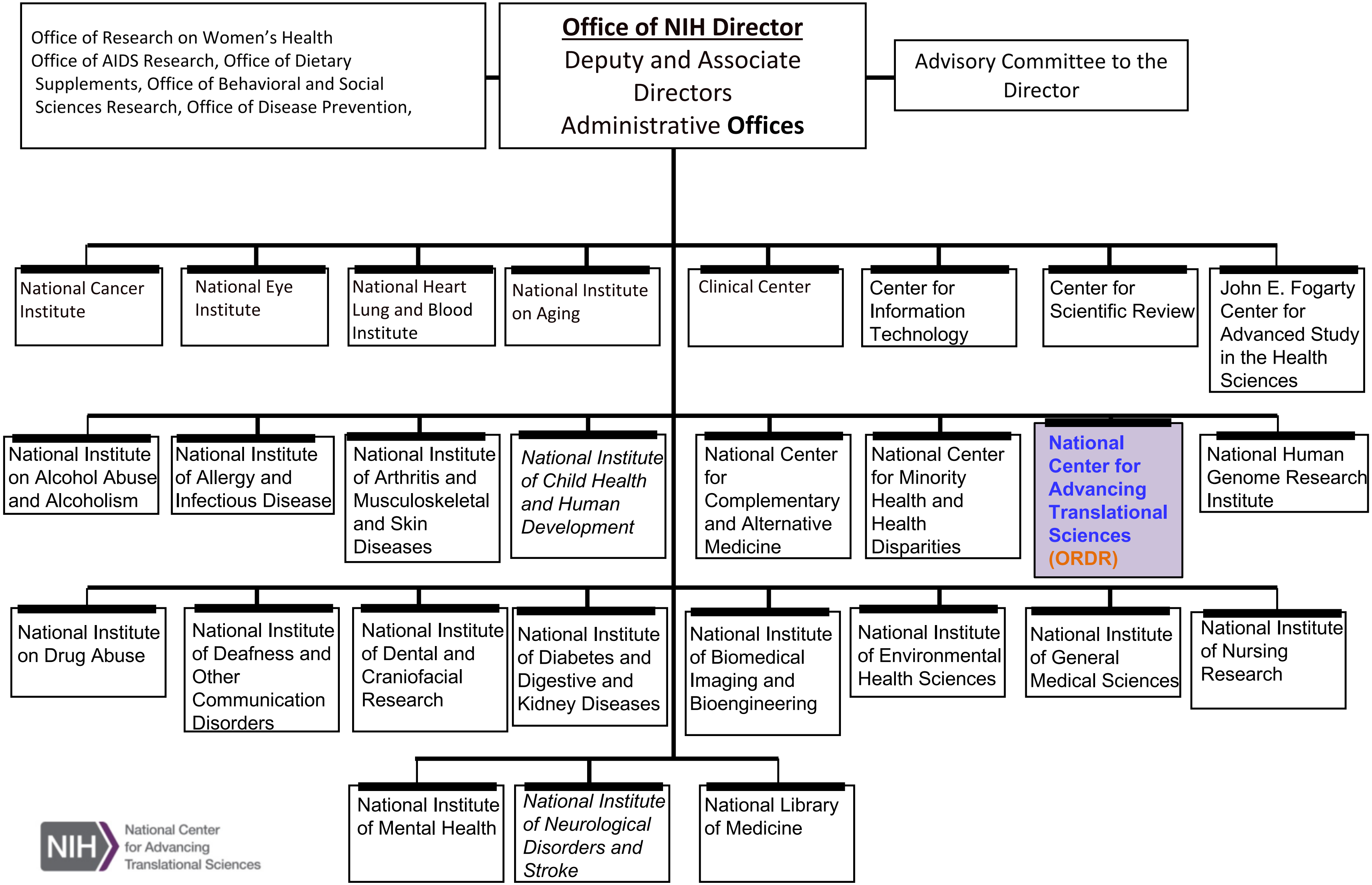
NIH National Center for Advancing Translational Sciences

Outline

- Office of Rare Diseases Research (ORDR), NCATS rare diseases programs
  - RDCRN Program
  - Other programs

- Resources and Opportunities at NCATS for translational research

A View of the National Institutes of Health (NIH), USA



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Office of AIDS Research, Office of Dietary Supplements, Office of Behavioral and Social Sciences Research, Office of Disease Prevention,

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- National Cancer Institute
- National Eye Institute
- National Heart Lung and Blood Institute
- National Institute on Aging
- Clinical Center
- Center for Information Technology
- Center for Scientific Review
- John E. Fogarty Center for Advanced Study in the Health Sciences

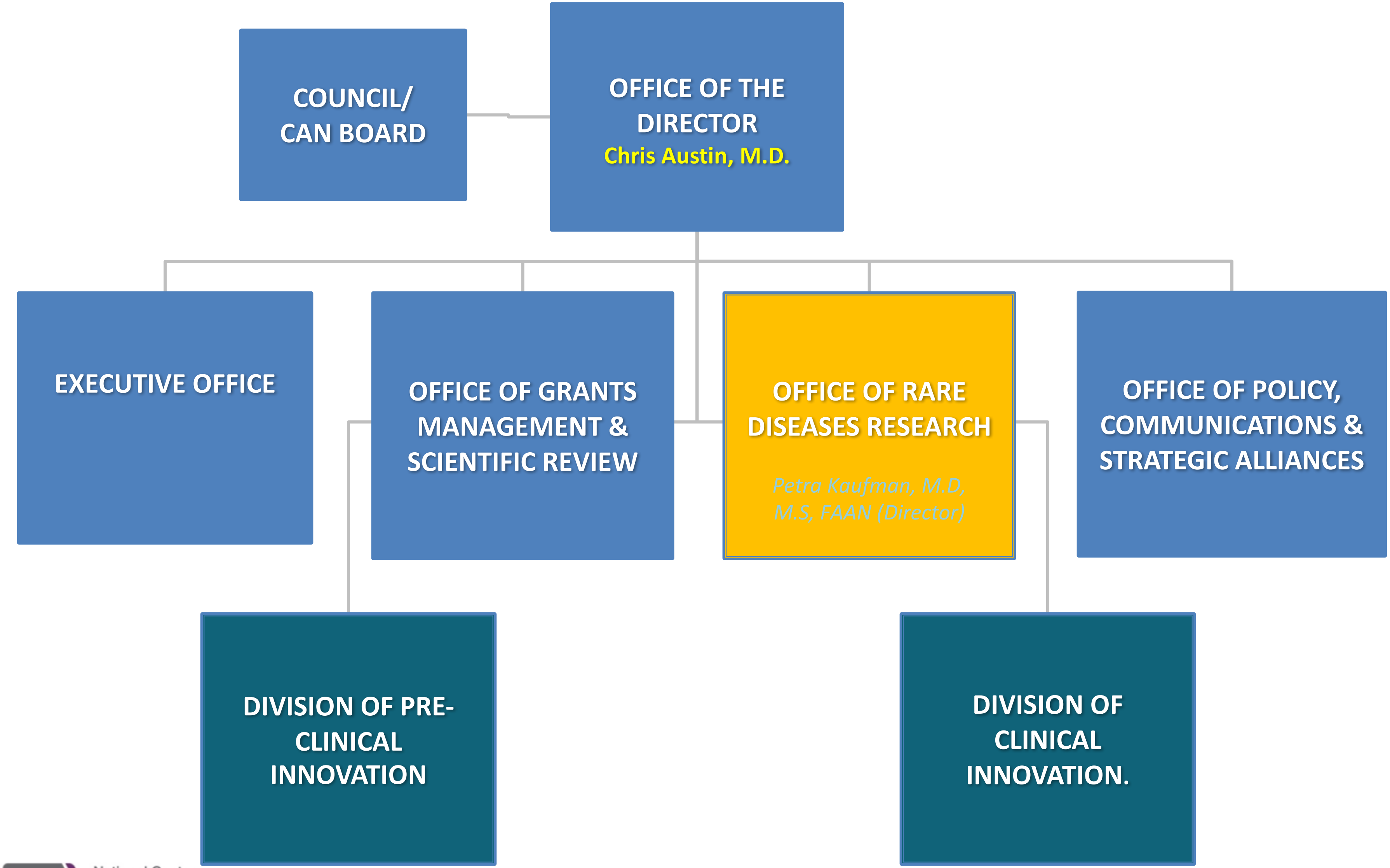
- National Institute on Alcohol Abuse and Alcoholism
- National Institute of Allergy and Infectious Disease
- National Institute of Arthritis and Musculoskeletal and Skin Diseases
- *National Institute of Child Health and Human Development*
- National Center for Complementary and Alternative Medicine
- National Center for Minority Health and Health Disparities
- **National Center for Advancing Translational Sciences (ORDR)**
- National Human Genome Research Institute

- National Institute on Drug Abuse
- National Institute of Deafness and Other Communication Disorders
- National Institute of Dental and Craniofacial Research
- National Institute of Diabetes and Digestive and Kidney Diseases
- National Institute of Biomedical Imaging and Bioengineering
- National Institute of Environmental Health Sciences
- National Institute of General Medical Sciences
- National Institute of Nursing Research

- National Institute of Mental Health
- *National Institute of Neurological Disorders and Stroke*
- National Library of Medicine

NIH National Center for Advancing Translational Sciences

National Center for Advancing Translational Sciences (NCATS)



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DIVISION OF
CLINICAL
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Office of Rare Diseases Research (ORDR), NCATS Collaborative Programs/Initiatives

Stimulates and coordinates research on rare diseases

- **Rare Diseases Clinical Research Network (RDCRN) Program**
- Genetic and Rare Diseases Information Center (GARD)
- Scientific Conferences
- Participation in Bench to Bedside program
- Global Rare Diseases Registry and Repository (GRDR)

Rare Diseases: Background

Prevalence < 200,000 people

in the USA

> 6,500 rare diseases

30 M affected in US

Many undiagnosed

< 500 have any treatment

Challenges for Rare Diseases Research

- Disease often not well characterized or defined
- Rarity means:
  - Recruitment for trials is usually quite difficult
  - Study populations become widely dispersed
  - Few expert centers for diagnosis, management, and research
- Often little high-quality evidence available to guide treatment

RARE DISEASES CLINICAL RESEARCH NETWORK

Initiative of the National Center for Advancing Translational Sciences (NCATS)

RDCRN Program at ORDR, NCATS

- Background
- Goals
- Collaborative partners
- Special features of the program
- Examples of successful collaborative efforts

Rare Diseases Clinical Research Network (RDCRN) Program:

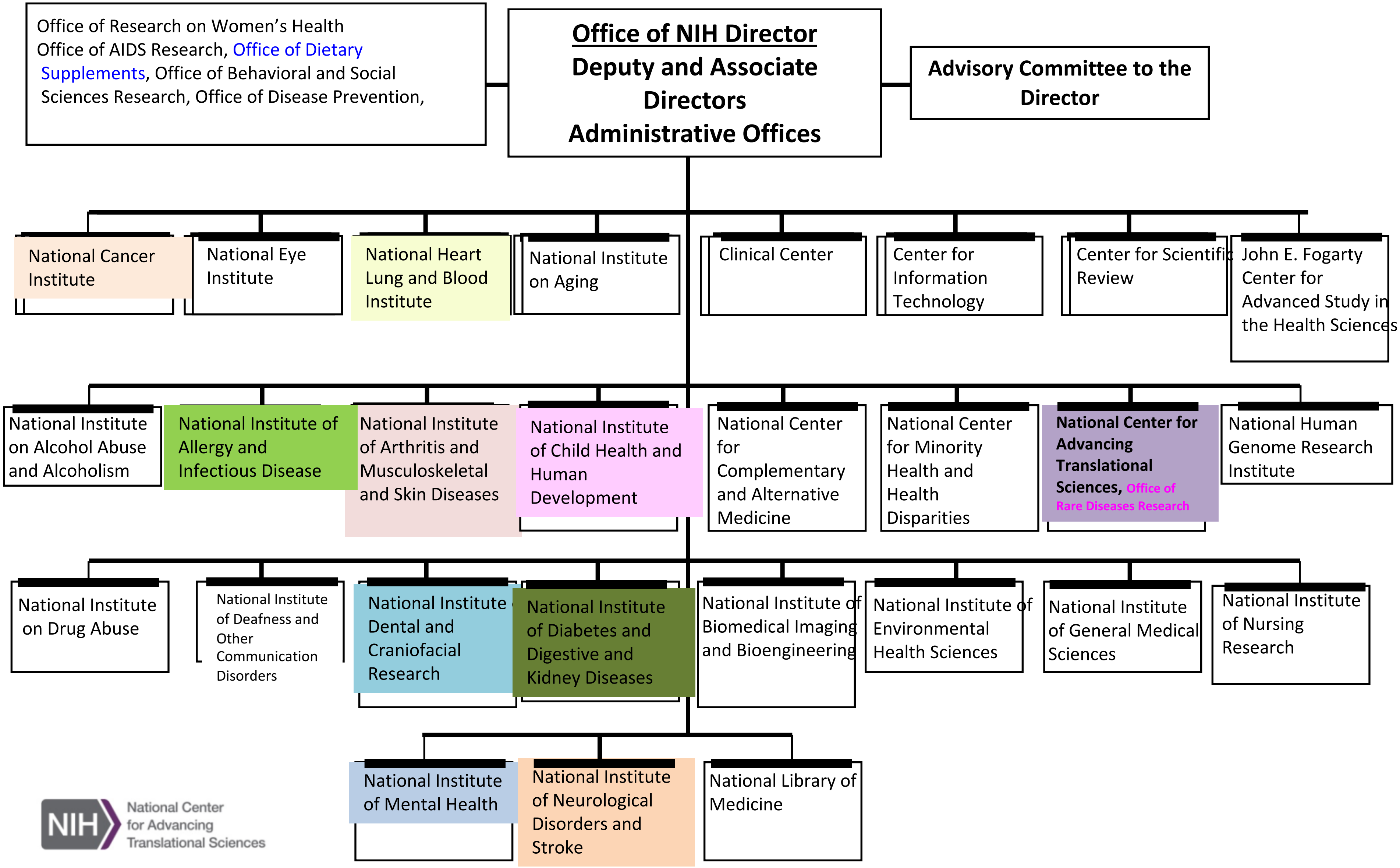
A Working Model for Collaboration and Partnership with Patients and Advocacy Groups to Facilitate Research Efforts

1) RDCRN Program: Established by the Office of Rare Diseases Research (ORDR)

Rare Diseases Clinical Research Network

Initiative of the National Center for Advancing Translational Sciences (NCATS)

RDCRN Program : Led by NCATS (ORDR), Collaboration with 10 NIH Institutes



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National Institute of Child Health and Human Development

National Center for Complementary and Alternative Medicine

National Center for Minority Health and Health Disparities

National Center for Advancing Translational Sciences, **Office of Rare Diseases Research**

National Human Genome Research Institute

National Institute on Drug Abuse

National Institute of Deafness and Other Communication Disorders

National Institute Dental and Craniofacial Research

National Institute of Diabetes and Digestive and Kidney Diseases

National Institute of Biomedical Imaging and Bioengineering

National Institute of Environmental Health Sciences

National Institute of General Medical Sciences

National Institute of Nursing Research

National Institute of Mental Health

National Institute of Neurological Disorders and Stroke

National Library of Medicine

NIH National Center for Advancing Translational Sciences

RDCRN Program: Background Information

- Established (in 2003 by ORDR) in response to a Request for Application (RFA). Ten consortia a central Data Management and Coordinating Center (DMCC)
- Expanded in 2009 to 17 consortia and a DMCC (Reissuance of RFA)
- Each RDCRN Consortium : multiple diseases/ investigators / sites, collaborative clinical research Involving Patient Advocacy Groups (PAGs) <u>as research partners</u>

RDCRN Program: Background Information

- *These are cooperative agreement (U54) awards for 5 years*. Scientific collaborators (project scientists) from ORDR, NCATS and NIH Institutes/Centers (ICs)
- Each awardee (Consortium) receives no more than $1.25 M Total Cost/year for multi site studies
- RDCRN 3rd cycle (Renewed – 2014), an ORDR, NCATS Initiative

  22 distinct multi-site Consortia and a DMCC

  A Network of Networks!

Goals of the RDCRN Program

- Facilitate clinical research by:
  - Creating multi-site Consortia focused on a group of at least three related diseases
  - Making meaningful large-scale clinical studies possible
    - Longitudinal studies, Clinical Trials, Natural History Studies are required
    - Establishing uniform protocols for data collection
    - Cost sharing infrastructure
- Collaborate with patients advocacy groups (as research partners), DMCC and NIH scientific staff
- Train new investigators
- Support Pilot Projects Program
- Provide Website resource for education and research in rare diseases

Requirements

About RDCRN Program

- Collectively, the RDCRN is studying 200 rare diseases in natural history and clinical trials at 418 active clinical sites located in the US and in 24 countries.
- There are more than 90 active protocols.
- 41,519 patients have enrolled in clinical studies.
- There have been 265 trainees.
- There are 3,545 collaborative consortium members.
- There are 144 PAGs as research partners, collectively formed a Coalition (RDCRN-CPAG).

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

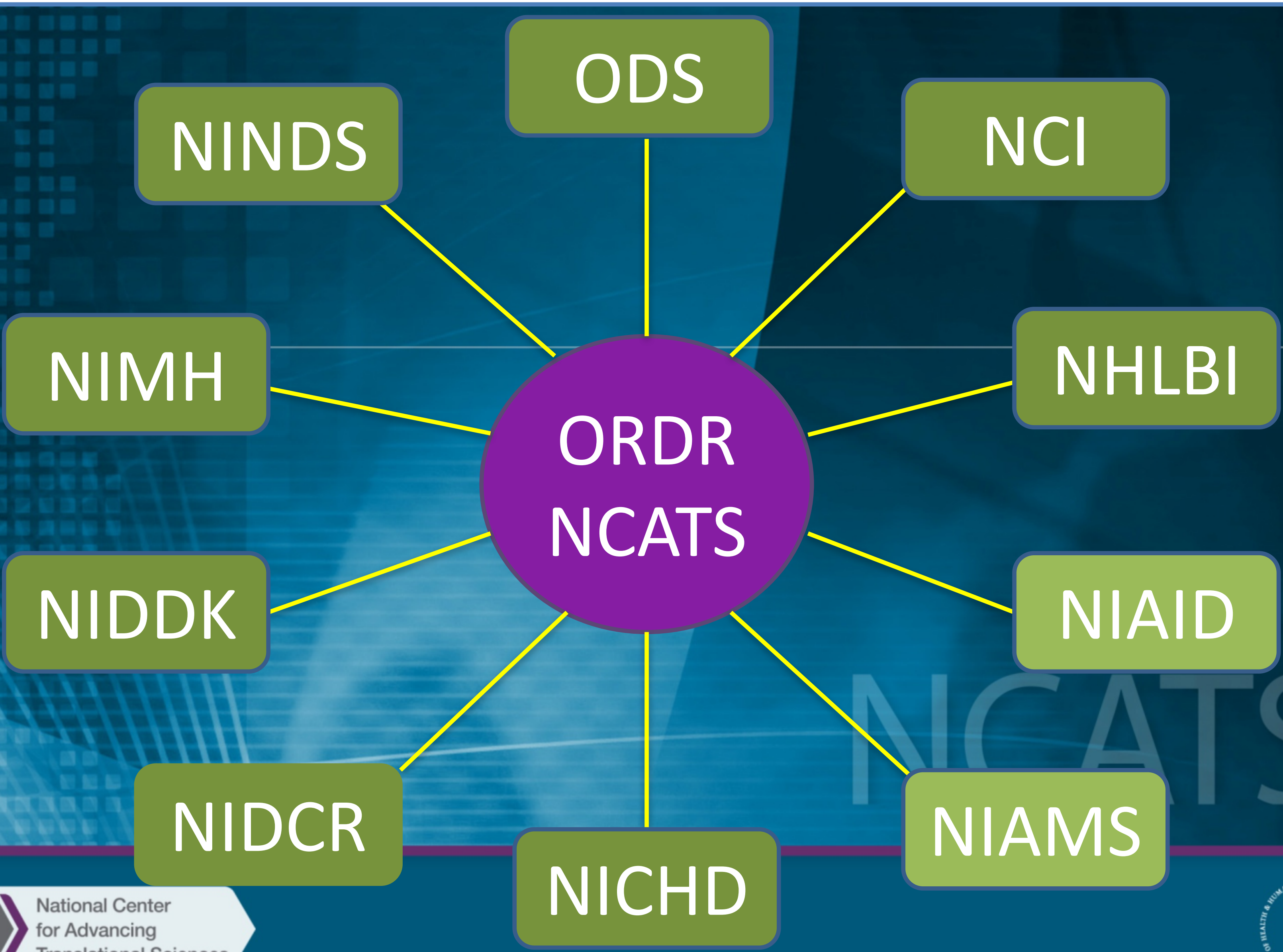
RDCRN Program: Special Features

- *The RDCRN is unique in its approach to addressing* rare diseases as a group. Each consortium studies a group of *minimum three related rare diseases*.

- The direct involvement of PAGs as research partners *is a major feature and requirement of this network*.

- NCATS (ORDR program)—Collaboration with 10 NIH ICs
    - ➢ Cooperative Agreement Awards managed by collaborating NIH IC (*culture change*)

RDCRN Program at NCATS



ORDR
NCATS

ODS

NCI

NHLBI

NIAID

NIAMS

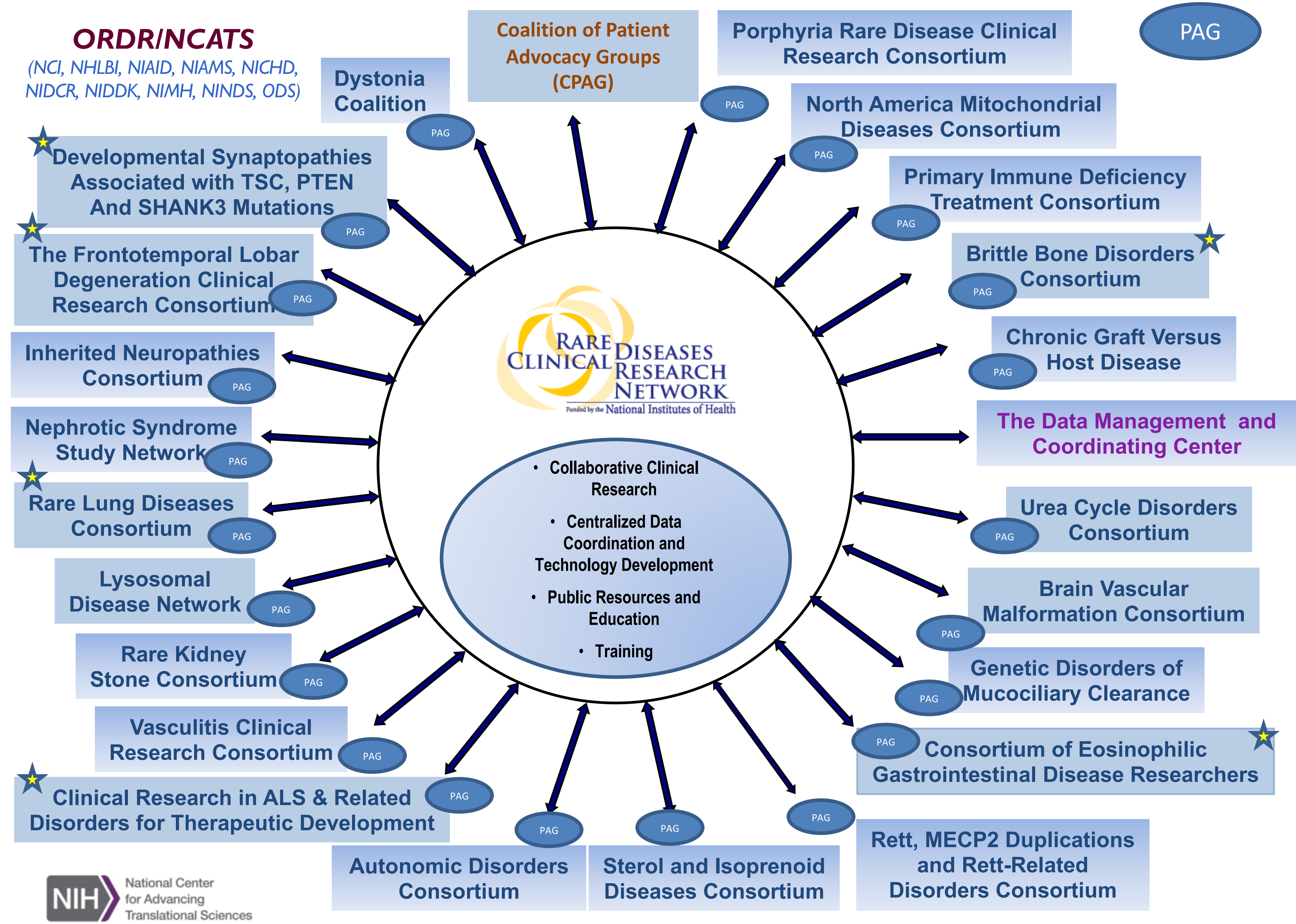
NICHD

NIDCR

NIDDK

NIMH

NINDS



ORDR/NCATS

(NCI, NHLBI, NIAID, NIAMS, NICHD, NIDCR, NIDDK, NIMH, NINDS, ODS)

PAG

Dystonia Coalition
PAG

Coalition of Patient Advocacy Groups (CPAG)

Porphyria Rare Disease Clinical Research Consortium
PAG

North America Mitochondrial Diseases Consortium
PAG

Primary Immune Deficiency Treatment Consortium
PAG

★ Brittle Bone Disorders Consortium
PAG

Chronic Graft Versus Host Disease
PAG

The Data Management and Coordinating Center

Urea Cycle Disorders Consortium
PAG

Brain Vascular Malformation Consortium
PAG

Genetic Disorders of Mucociliary Clearance
PAG

★ Consortium of Eosinophilic Gastrointestinal Disease Researchers
PAG

Rett, MECP2 Duplications and Rett-Related Disorders Consortium
PAG

Sterol and Isoprenoid Diseases Consortium
PAG

Autonomic Disorders Consortium
PAG

★ Clinical Research in ALS & Related Disorders for Therapeutic Development
PAG

Vasculitis Clinical Research Consortium
PAG

Rare Kidney Stone Consortium
PAG

Lysosomal Disease Network
PAG

★ Rare Lung Diseases Consortium
PAG

Nephrotic Syndrome Study Network
PAG

Inherited Neuropathies Consortium
PAG

★ The Frontotemporal Lobar Degeneration Clinical Research Consortium
PAG

★ Developmental Synaptopathies Associated with TSC, PTEN And SHANK3 Mutations
PAG

RARE DISEASES CLINICAL RESEARCH NETWORK
Funded by the National Institutes of Health

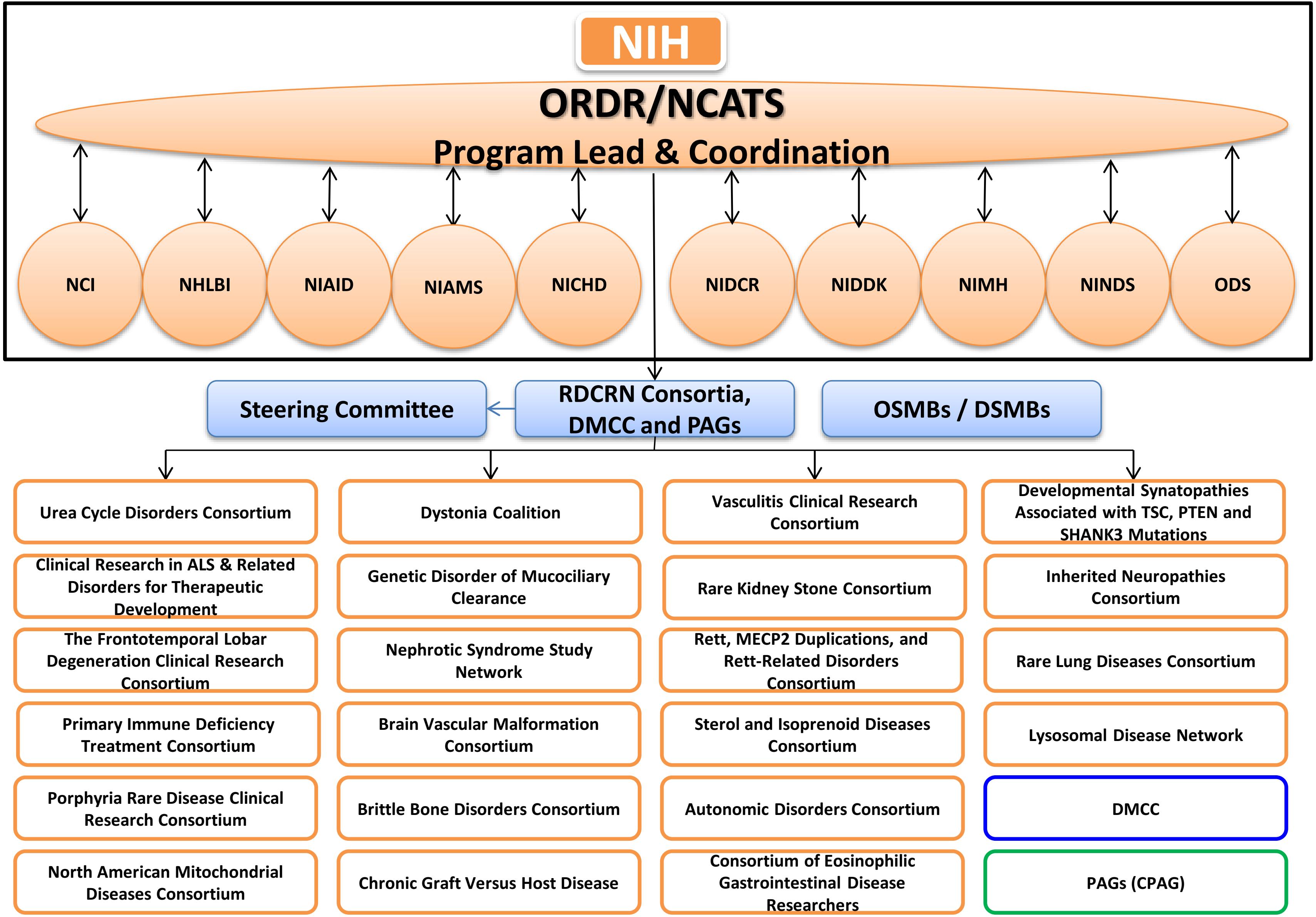
- Collaborative Clinical Research
- Centralized Data Coordination and Technology Development
- Public Resources and Education
- Training

NIH National Center for Advancing Translational Sciences

RDCRN Protocols

Type of Study	Number of Protocols
Pilot	23
Longitudinal	91
Phase I	6
Phase II	18
Phase III	3
Cross Sectional	7
Industry Sponsored	12
Case Control	6
Chart Review	4

Rare Disease Clinical Research Network 3 (RDCRN 3)



NIH

ORDR/NCATS

Program Lead & Coordination

NCI

NHLBI

NIAID

NIAMS

NICHD

NIDCR

NIDDK

NIMH

NINDS

ODS

Steering Committee

RDCRN Consortia, DMCC and PAGs

OSMBs / DSMBs

Urea Cycle Disorders Consortium

Dystonia Coalition

Vasculitis Clinical Research Consortium

Developmental Synatopathies Associated with TSC, PTEN and SHANK3 Mutations

Clinical Research in ALS & Related Disorders for Therapeutic Development

Genetic Disorder of Mucociliary Clearance

Rare Kidney Stone Consortium

Inherited Neuropathies Consortium

The Frontotemporal Lobar Degeneration Clinical Research Consortium

Nephrotic Syndrome Study Network

Rett, MECP2 Duplications, and Rett-Related Disorders Consortium

Rare Lung Diseases Consortium

Primary Immune Deficiency Treatment Consortium

Brain Vascular Malformation Consortium

Sterol and Isoprenoid Diseases Consortium

Lysosomal Disease Network

Porphyria Rare Disease Clinical Research Consortium

Brittle Bone Disorders Consortium

Autonomic Disorders Consortium

DMCC

North American Mitochondrial Diseases Consortium

Chronic Graft Versus Host Disease

Consortium of Eosinophilic Gastrointestinal Disease Researchers

PAGs (CPAG)

NIH National Center for Advancing Translational Sciences

(RDCRN-CPAG)

A model of community engagement to facilitate clinical research

A unique feature of the RDCRN is the ***direct engagement*** *of the rare disease patient community through* ***PAGs working as research partners with individual consortium*** *of RDCRN, and collectively as the Coalition Patient Advocacy Groups (CPAG) comprised of all PAGs of RDCRN.*

The importance of PAGs looking on the issues not just specific to their diseases of interest but across rare diseases.

Value of PAGs as Research Partners

Since 2004 PAGs within RDCRN are involved in more than one of the following roles as research partners-

- Recruit patients for clinical studies, encourage participation in NHS
- Identify cohorts of patients with range of phenotypic expression
- Educate patients, public, media and health care providers
- Identify research efforts and translate research results to communities

Value of PAGs as Research Partners

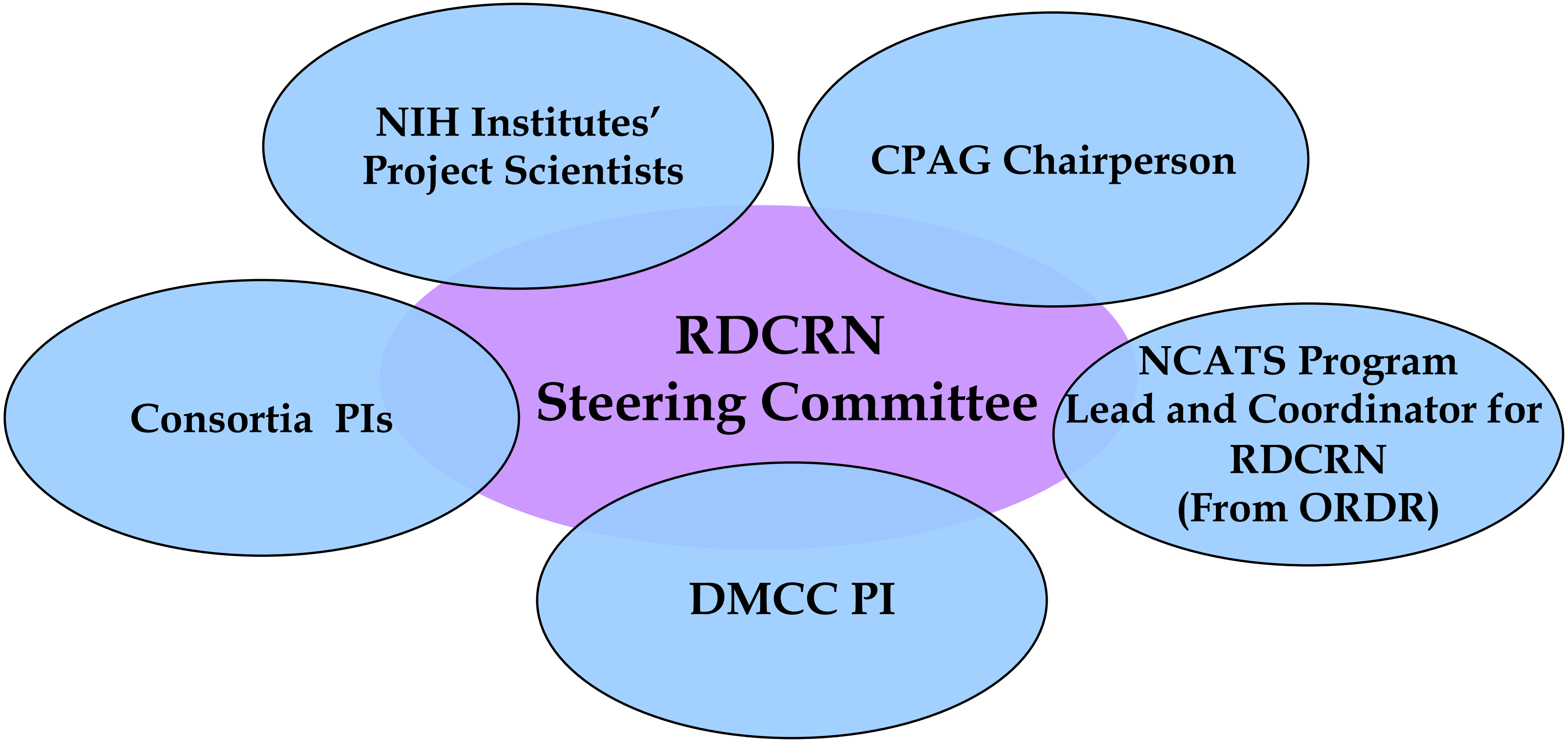
- Organize and fund research based Scientific conferences and meetings for patients/families/caregivers
- Provide financial support for research and training programs of RDCRN (consortia) and patient registries
- Provide financial support for *travel clinics* to facilitate patient access to investigators and studies
- Establish global partnership

RDCRN International Sites

- Argentina
- Australia
- Austria
- Belgium
- Brazil
- Canada
- China
- Denmark
- England
- France
- Germany
- Iceland
- Ireland
- Israel
- Italy
- Japan
- Korea
- Mexico
- Netherlands
- Russia
- Scotland
- Spain
- *Switzerland*
- Turkey

RDCRN Steering Committee Organization

(Review, facilitate and establish all Network procedures and functions)



NIH Institutes' Project Scientists

CPAG Chairperson

RDCRN Steering Committee

Consortia PIs

NCATS Program Lead and Coordinator for RDCRN (From ORDR)

DMCC PI

RDCRN PAGs and Principal Investigaotrs Manuscript

“The Partnership of Patient Advocacy Groups and Clinical Investigators in the Rare Diseases Clinical Research Network” has been published in Orphanet Journal of Rare Diseases.

Ojrd.biomedcentral.com (May 18th, 2016)

Constant Communication for Collaboration!

- Monthly RDCRN Steering Committee calls, two in person meetings in Washington DC area (*CPAG chair*)
- Monthly meeting with NIH Institutes~50 medical officer and program officers
- Biweekly meetings with RDCRN-DMCC
- Quarterly calls with *RDCRN-CPAG*, in person
- Individual RDCRN-Consortium calls with *PAG's participation*

RDCRN-Data Management and Coordinating Center (DMCC)

- Supports RDCRN by providing technologies, tools to collect clinical research data and support for study design and data analysis
- On-line protocol management system
  - Web-based patient enrollment (recruitment and referral)
  - Data entry and collection with data standards
  - Adverse event reporting
- Provides protocol training for research staff

Responsibilities of RDCRN-DMCC (Cont.)

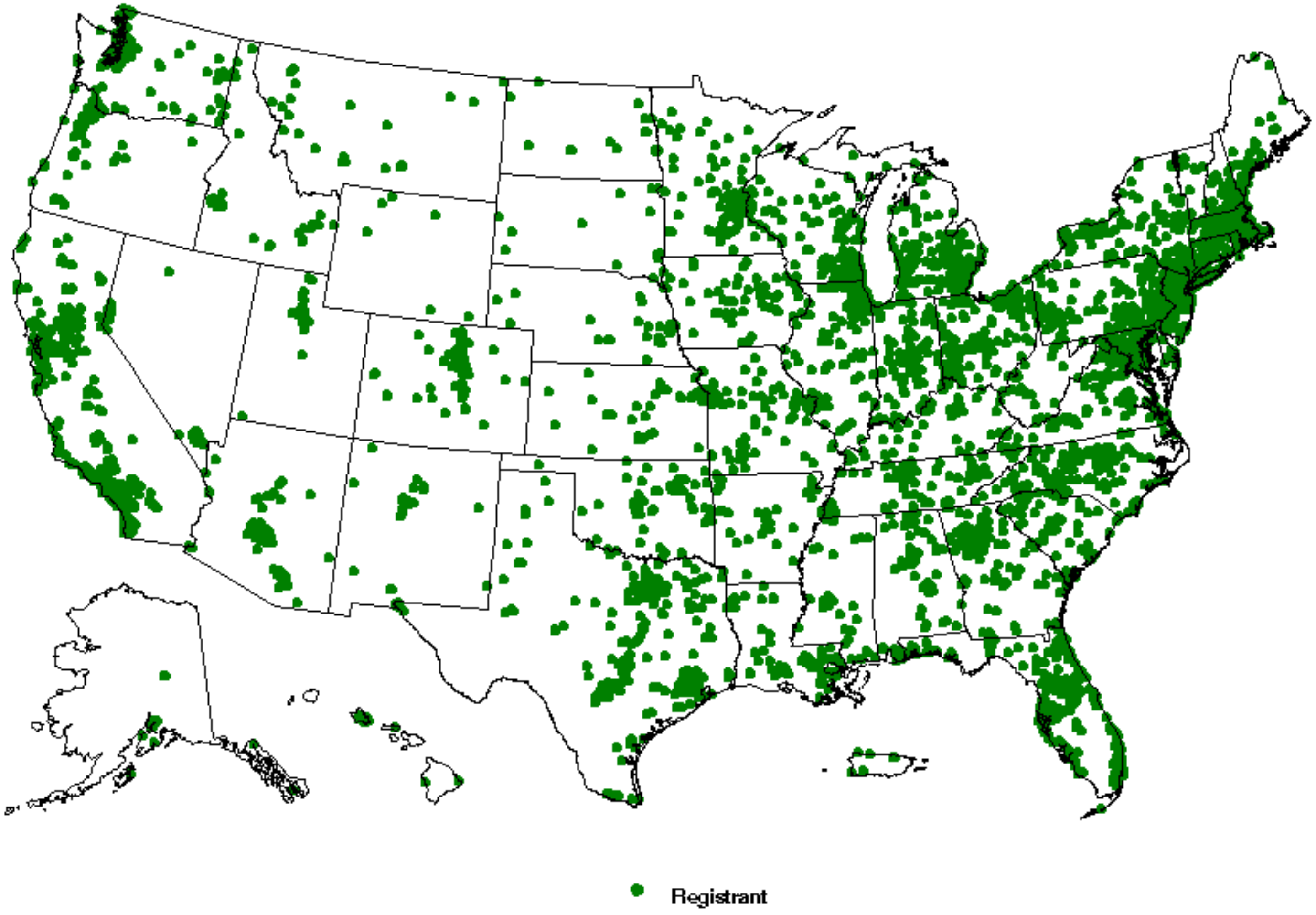
- Works with the individual NIH Institutes’ Data and Safety Monitoring Boards to establish protocols for Adverse Events notification and reporting
- Monitor Network protocol adherence, data collection and data submission
- Coordinates site visits for auditing individual consortia sites

Responsibilities of RDCRN DMCC (Cont.)

- Provides a user-friendly web resource site for the public, research scientists, and clinicians; *involvement of PAGs* (>2 million hits/year)
- Maintain members’ website, documentation and database
- *Oversees and maintains RDCRN Patient Contact Registry*

RDCRN Contact Registry (2004)

(U.S. Geographic Distribution of Contact Registrants)



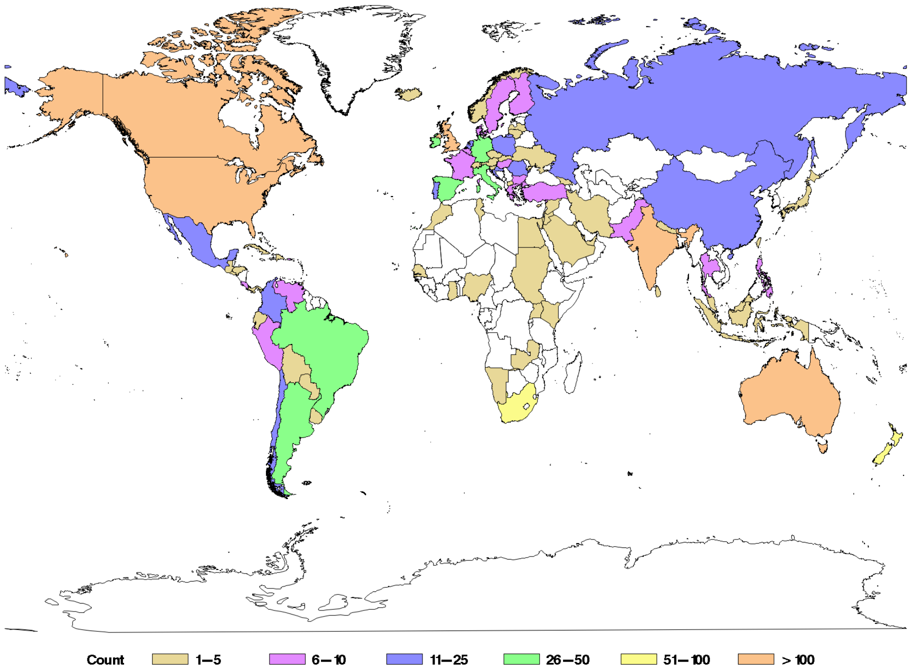
-Enrollment open to patients with diseases under study by Consortia

- Provides international on-line system for communication, recruitment, research

-

RDCRN Contact Registry Overview

Data as of October 29th, 2016



- 213 diseases represented
- 116 countries
- 19,144 total registrations
- 38% referred from PAGs
- 38% from internet
- 7% referred from med. prof.
- 260,000+ email communications

Goals:
To inform registrants about RDCRN studies available;
To disseminate information about RDCRN activities

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Data Sharing
(RDCRN Data Repository)

- The RDCRN-DMCC also coordinates with ORDR program staff including registration with and data uploading of appropriate RDCRN studies to ORDR-governed data repository
- Through dbGaP, a database for genotypes and phenotypes (NCBI, National Library of Medicine)
- Data transfer to dbGaP occurs on regular basis
- RDCRN Data Access Committee (DAC)

Partnering for Success

Patient Groups

Academics

Industry

Government (in US: NIH, FDA)

An Example of Collaboration/Scientific Advancements: The Urea Cycle Disorders Consortia at Children's National Medical Center

- 19 Academic Research Centers in USA and 2 International Sites
- Collaborators With European Registry And Network For Intoxication Type Metabolic Disorders (EIMD)
- Industry Partnerships - *3 Products Approved*
  - ➢ Ucyclyd Pharma: Ammonul
  - ➢ Recordati: Carbaglu
  - ➢ Hyperion: Ravicti

➢ Patient Advocacy Group – The National Urea Cycle Disorders Foundation

➢ Foundations - O'Malley Family Foundation, Kettering Fund, Rotenberg Family Foundation, and Dietmar-Hopp Foundation

➢ ORDR/NCATS and NICHD (from NIH), providing support and scientific collaboration

Another Example: RDCRN-Rare Lung Diseases Consortium (RLDC)

- In early 2015 FDA accepted for priority review a supplemental New Drug Application for (sNDA) RAPAMUNE® for the treatment of lymphangioleiomyomatosis (LAM)
- LAM is a rare, progressive lung disease that primarily affects women of childbearing age that is often fatal. (March/2015 FDA approval)
- *This is the first drug approved for the treatment of LAM!*
- This is an accomplishment of the Multicenter International LAM Efficacy and Safety of Sirolimus (MILES) Trial (conducted by Dr. Francis McCormack of RDCRN RLDC in collaboration with LAM Foundation). The sNDA was based on results from the MILES Trial. (Wyeth)
  - *Collaborative effort!*

Genetic Disorders of Mucociliary Clearance Consortium & Primary Ciliary Diskinesia (PCD) Foundation

- Diagnosis, monitoring, and treatment of primary ciliary dyskinesia: PCD foundation consensus recommendations based on state of the art review.
- Pediatric Pulmonology. September 29, 2015
- Shapiro AJ, Zariwala MA, Ferkol T, Davis SD, Sagel SD, Dell SD, Rosenfeld M, Olivier KN, Milla C, Daniel SJ, Kimple AJ, *Manion M*, Knowles MR, Leigh MW

Through the RDCRN program……

- New diagnostic methods have been generated
- New gene identification has been facilitated and
- New therapies have been identified.

by creating *collaborative multidisciplinary, multi-site research consortia* consisting of PAGs, academic researchers from domestic and international sites and project scientists from NCATS & NIH ICs as collaborators, the program has demonstrated that collaborative effort can accelerate clinical research.

Team Work!

- Clinical Sites
- Principal and co-investigators and the DMCC (multidisciplinary group)
- Trainees
- Study Coordinators
- *Patient Advocacy Groups (PAGs)*
- Pharmaceutical industry
- ORDR/NCATS and NIH Institutes staff (program officers and project scientists)
- *Patients*

RDCRN: Working model for collaborative, multi-site clinical studies with PAGs partnership in an inexpensive way!

RDCRN consists of 418 sites, 144 PAGs and conducts research on more than 200 rare diseases

T. E. A. M.

RDCRN: *An Effective and Working Model for Collaborative Multi-Site Rare Diseases Research Efforts*

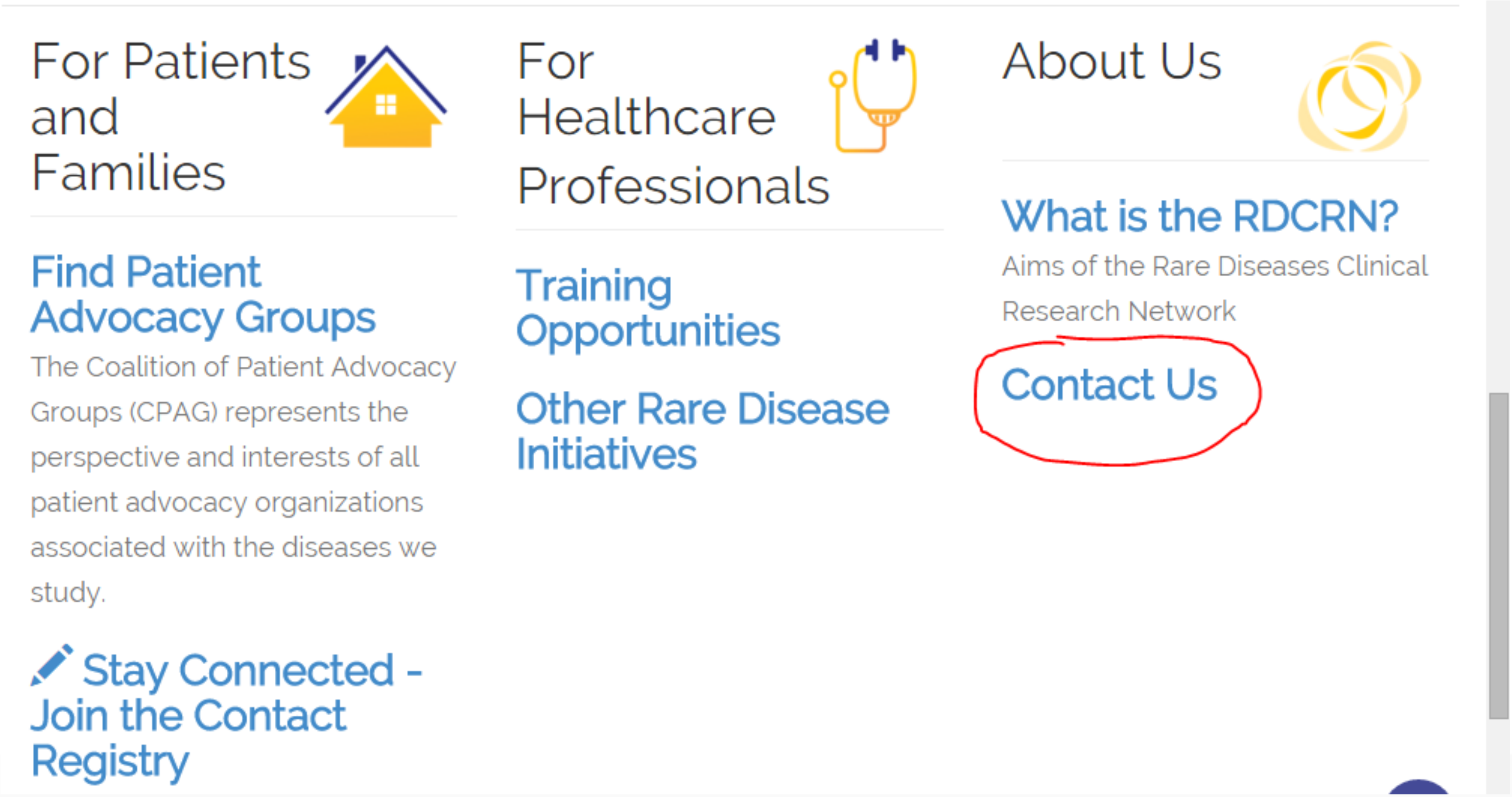
Rare Diseases Clinical Research Network

Initiative of the National Center for Advancing Translational Sciences (NCATS)

RDCRN Website

https://www.rarediseasesnetwork.org/

RDCRN Homepage



For Patients and Families

Find Patient Advocacy Groups

The Coalition of Patient Advocacy Groups (CPAG) represents the perspective and interests of all patient advocacy organizations associated with the diseases we study.

Stay Connected - Join the Contact Registry

For Healthcare Professionals

Training Opportunities

Other Rare Disease Initiatives

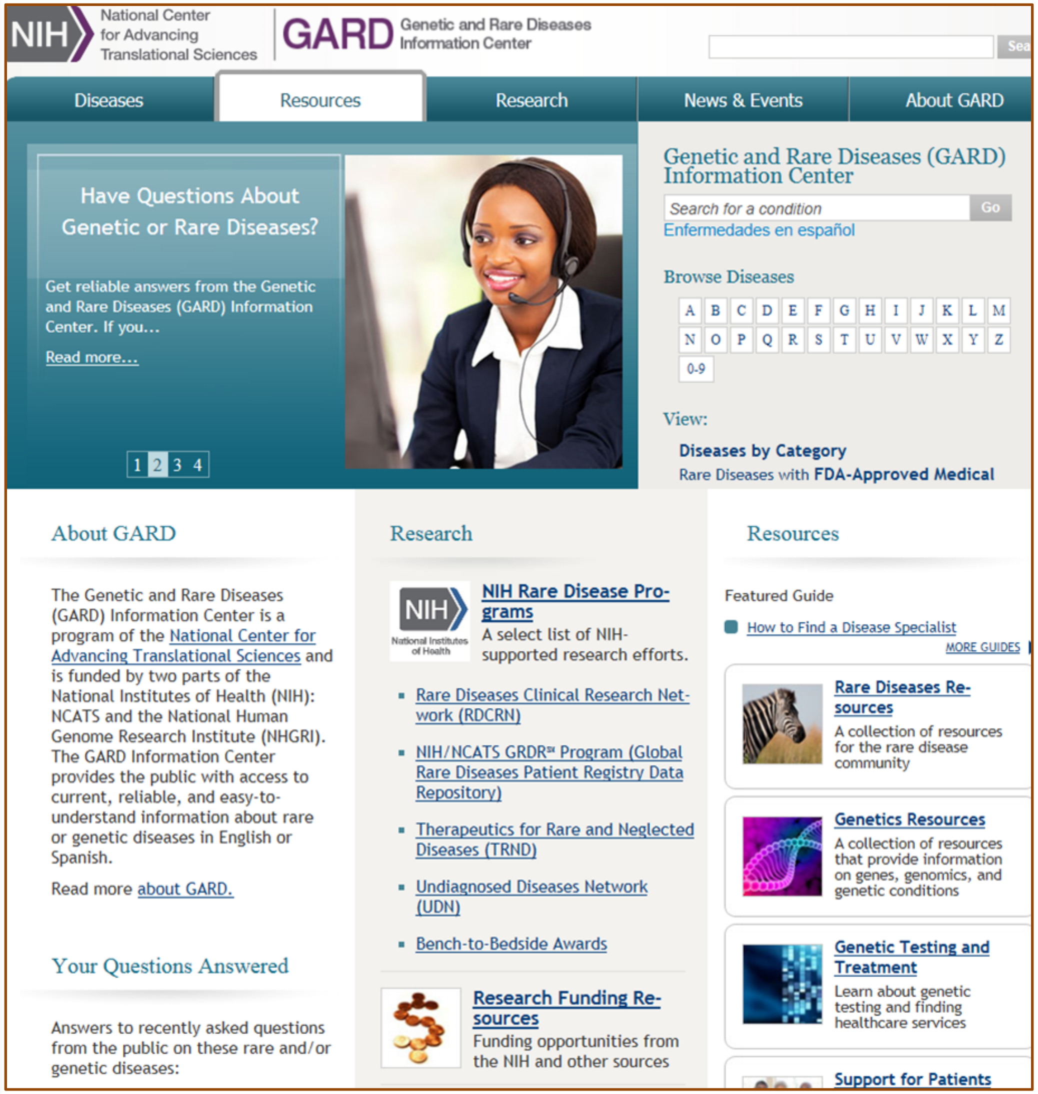
About Us

What is the RDCRN?

Aims of the Rare Diseases Clinical Research Network

Contact Us

2) NCATS Genetic and Rare Diseases Information Center (GARD)



Have Questions About Genetic or Rare Diseases?

Get reliable answers from the Genetic and Rare Diseases (GARD) Information Center. If you...

Read more...

Genetic and Rare Diseases (GARD) Information Center

Search for a condition

Enfermedades en español

Browse Diseases

View:

Diseases by Category

Rare Diseases with FDA-Approved Medical

About GARD

The Genetic and Rare Diseases (GARD) Information Center is a program of the National Center for Advancing Translational Sciences and is funded by two parts of the National Institutes of Health (NIH): NCATS and the National Human Genome Research Institute (NHGRI). The GARD Information Center provides the public with access to current, reliable, and easy-to-understand information about rare or genetic diseases in English or Spanish.

Read more about GARD.

Your Questions Answered

Answers to recently asked questions from the public on these rare and/or genetic diseases:

Research

NIH Rare Disease Programs

A select list of NIH-supported research efforts.

- Rare Diseases Clinical Research Network (RDCRN)
- NIH/NCATS GRDR℠ Program (Global Rare Diseases Patient Registry Data Repository)
- Therapeutics for Rare and Neglected Diseases (TRND)
- Undiagnosed Diseases Network (UDN)
- Bench-to-Bedside Awards

Research Funding Resources

Funding opportunities from the NIH and other sources

Resources

Featured Guide

- How to Find a Disease Specialist

Rare Diseases Resources

A collection of resources for the rare disease community

Genetics Resources

A collection of resources that provide information on genes, genomics, and genetic conditions

Genetic Testing and Treatment

Learn about genetic testing and finding healthcare services

Support for Patients

https://rarediseases.info.nih.gov

Online resource with:

- **Up-to-date, reliable** and **easy-to-understand information** on rare or genetic diseases
- Partnering with **Orphanet**
- In **English** or **Spanish**
- For **people** with **rare or genetic diseases**, their **families**, **friends**, **care providers** and wider communities
- **Contact information** for telephone and email queries

In addition……

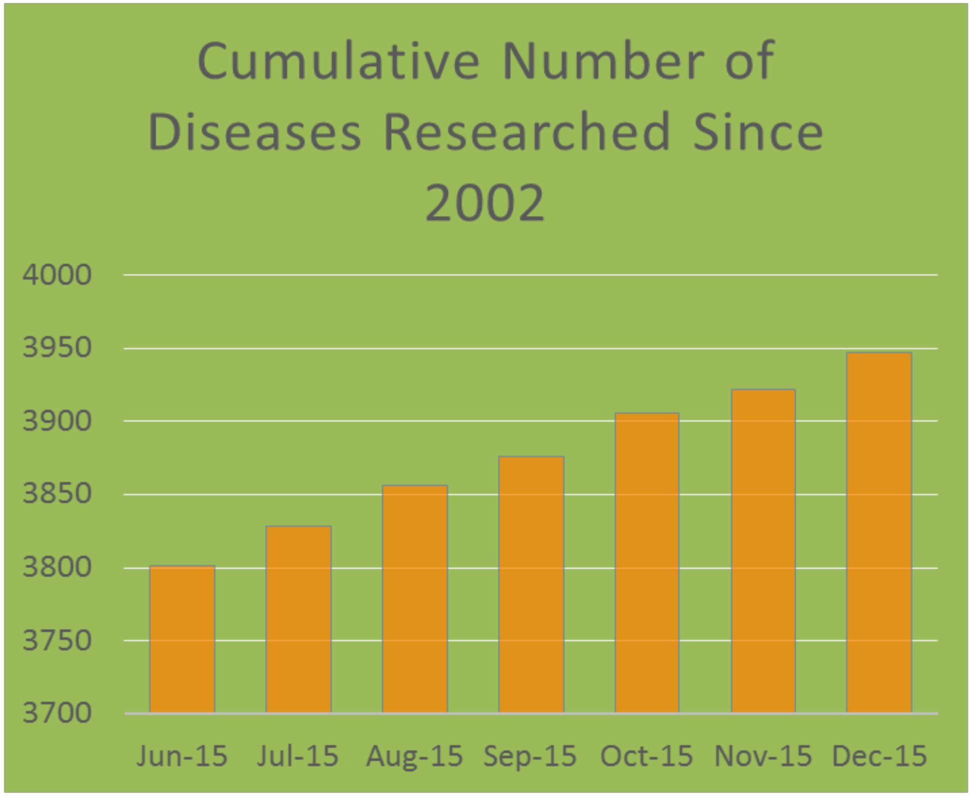
Specialized information specialist staff

- Genetic counselors and clinical geneticist
- Services in English or Spanish

Medical advisors

- Cytogeneticist/molecular geneticist consultant
- Medical consultant for referrals (retired NIH expert)
- Medical geneticist, NIH consultant from NIGMS

INQUIRY STATASTICS



- Answered more than 55,000 questions
- Receive approximately 400 - 500 inquiries/month
- 77% from the United States; 13% International
- 3% of inquiries are in Spanish

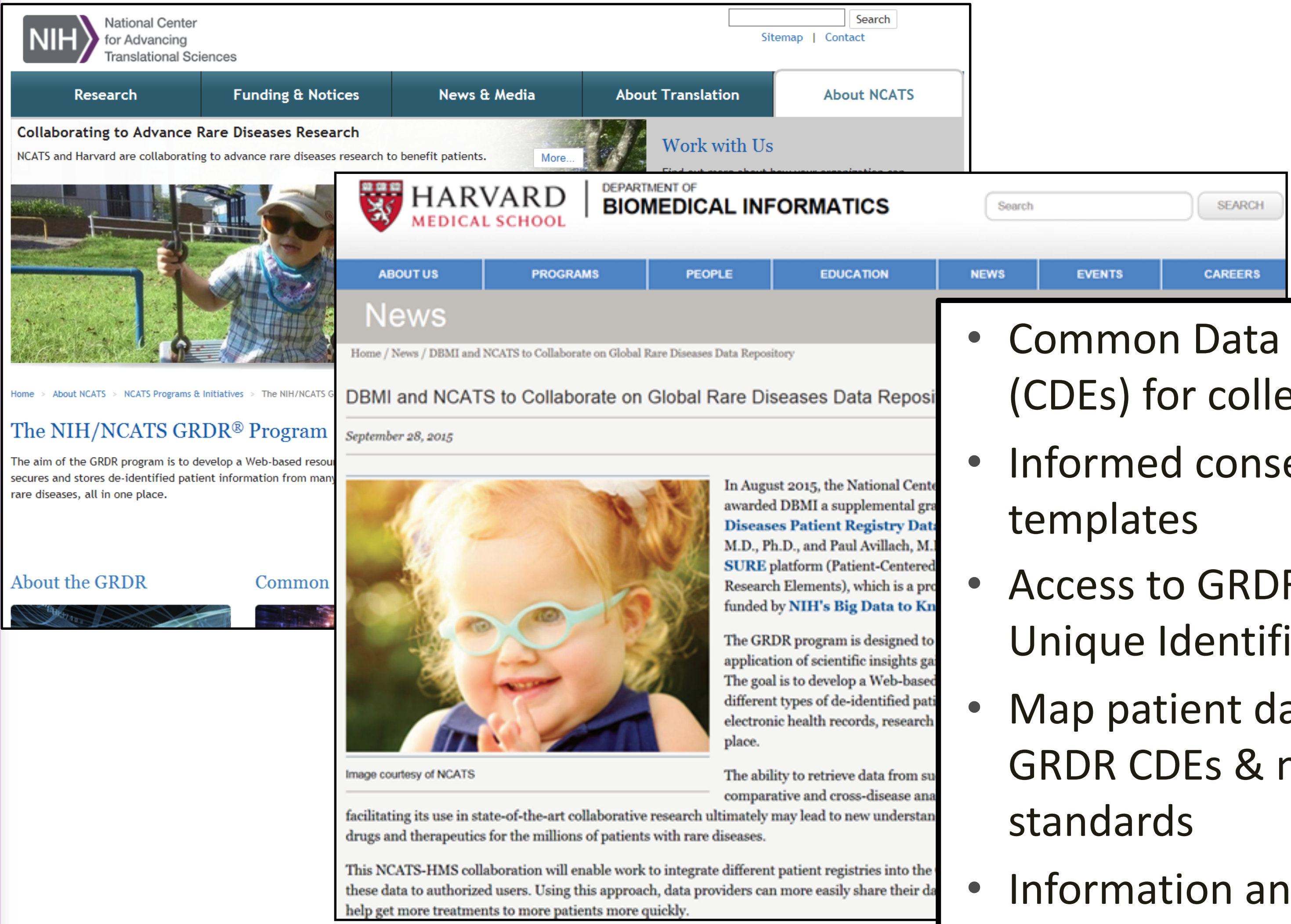
Answered questions on more

than 4000 diseases

3) Scientific Conferences Program

- Identify Research Opportunities
- Establish Research Priorities
- Develop Research Agenda (R13/U13 grant applications)
- ORDR can provide funding (1200 conferences supported)
- Webcast of many conference available

4) NIH/NCATS Global Rare Diseases Registry (GRDR®) Program



Collaborating to Advance Rare Diseases Research

NCATS and Harvard are collaborating to advance rare diseases research to benefit patients.

Work with Us

The NIH/NCATS GRDR® Program

The aim of the GRDR program is to develop a Web-based resou
secures and stores de-identified patient information from many
rare diseases, all in one place.

About the GRDR

Common

DBMI and NCATS to Collaborate on Global Rare Diseases Data Reposi

September 28, 2015

In August 2015, the National Cente
awarded DBMI a supplemental gra
Diseases Patient Registry Dat
M.D., Ph.D., and Paul Avillach, M.
SURE platform (Patient-Centered
Research Elements), which is a pro
funded by **NIH's Big Data to Kn**

The GRDR program is designed to
application of scientific insights ga
The goal is to develop a Web-based
different types of de-identified pati
electronic health records, research
place.

Image courtesy of NCATS

The ability to retrieve data from su
comparative and cross-disease ana
facilitating its use in state-of-the-art collaborative research ultimately may lead to new understan
drugs and therapeutics for the millions of patients with rare diseases.

This NCATS-HMS collaboration will enable work to integrate different patient registries into the
these data to authorized users. Using this approach, data providers can more easily share their da
help get more treatments to more patients more quickly.

- Common Data Elements (CDEs) for collecting data
- Informed consent templates
- Access to GRDR Global Unique Identifier (GUID)
- Map patient data to GRDR CDEs & national standards
- Information and tools

Translational Research Program and Resources at NCATS, NIH

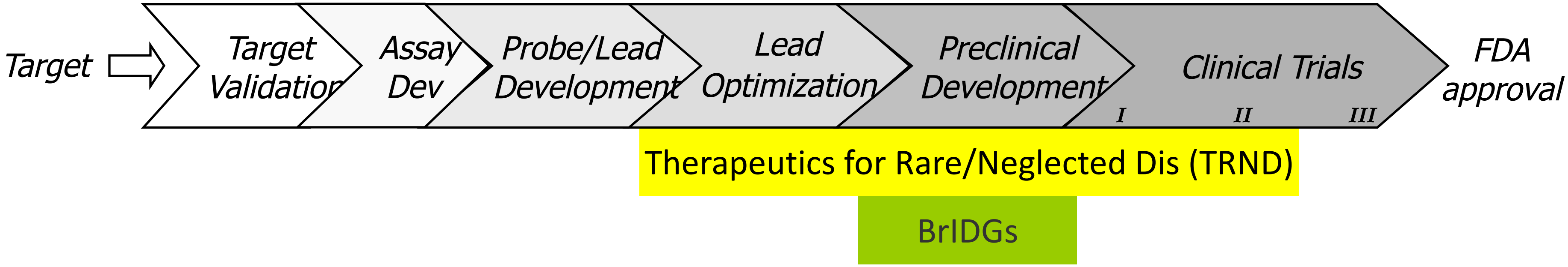
NCATS Scientific Initiatives

- **Preclinical Translational Science**
  - NCATS Chemical Genomics Center
  - *Therapeutics for Rare and Neglected Diseases program (TRND)*
  - *Bridging Interventional Development Gaps program (BriDGs)*
- **Clinical Translational Science**
  - *Clinical and Translational Science Awards*
  - ***Rare Disease Clinical Research Network program***
  - *New Therapeutic Uses program*
- **Re-engineering Translational Sciences**
  - Toxicology in the 21st Century
  - *Microphysiological Systems (Tissue Chip) program*

NCATS Therapeutics Development Programs

Therapeutics for Rare and Neglected Diseases (TRND)

Bridging Interventional Development Gaps (BrIDGs)



Model: Collaboration between NCATS labs with preclinical drug development expertise and external organizations with disease area/target expertise

Projects:

Disease must meet FDA Orphan or WHO NTD criteria

Entry from Probe to IND-enabling

Exit by adoption by external organization for completion of clinical development

Serve to develop new generally applicable platform technologies and paradigms

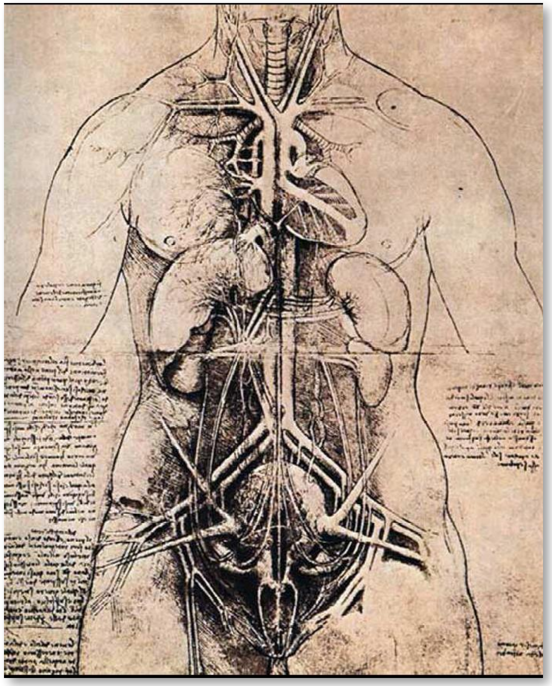
Eligible Collaborators:

Academic, Non-Profit, Government Lab, Biotech, Pharma

International applications accepted

Tissue Chip for Drug Screening Program

GOAL: Develop an *in vitro* platform that uses human tissues to evaluate the efficacy, safety and toxicity of promising therapies.



- All ten human physiological systems will be functionally represented by human tissue constructs:
  - Circulatory
  - Endocrine
  - Gastrointestinal
  - Immune
  - Integumentary
  - Musculoskeletal
  - Nervous
  - Reproductive
  - Respiratory
  - Urinary
- Physiologically relevant, genetically diverse, and pathologically meaningful
- Modular, reconfigurable platform
- Tissue viability for at least 4 weeks
- Community-wide access

NEW Tissue Chip for Drug Screening Program Funding Opportunity (Disease Modeling and Efficacy Testing Initiative)

- Goal: to create rare disease models that replicate pathology and other in vivo-like responses for testing effectiveness of candidate drugs
- Application receipt date (December 13th, 2016).
- RFA-TR-16-017. For questions contact Dan Tagle (NCATS)

NEW Tissue Chip for Drug Screening Program:

- Collaboration with Center for Advancement of Science in Space (CASIS)

- Purpose: to use tissue chip technology (leverage advances) at International Space Station, US, National Laboratory.

- Application receipt date (December 15th, 2016)

- RFA-TR-16-019.

Update Clinical Trials to Repurpose Drugs in Collaboration with E-Rare-3

- NCATS provide support for phase 1 and/or 2 clinical trial sites that will test the potential use of an existing molecule for the treatment of a rare disease as part of a multi-site international “E-Rare-3” trial.

- The ERA-NET “E-Rare” is coordinating research efforts of European countries in the field of rare diseases.

- Note: Collaboration with NCATS New Therapeutic Uses (NTU program)

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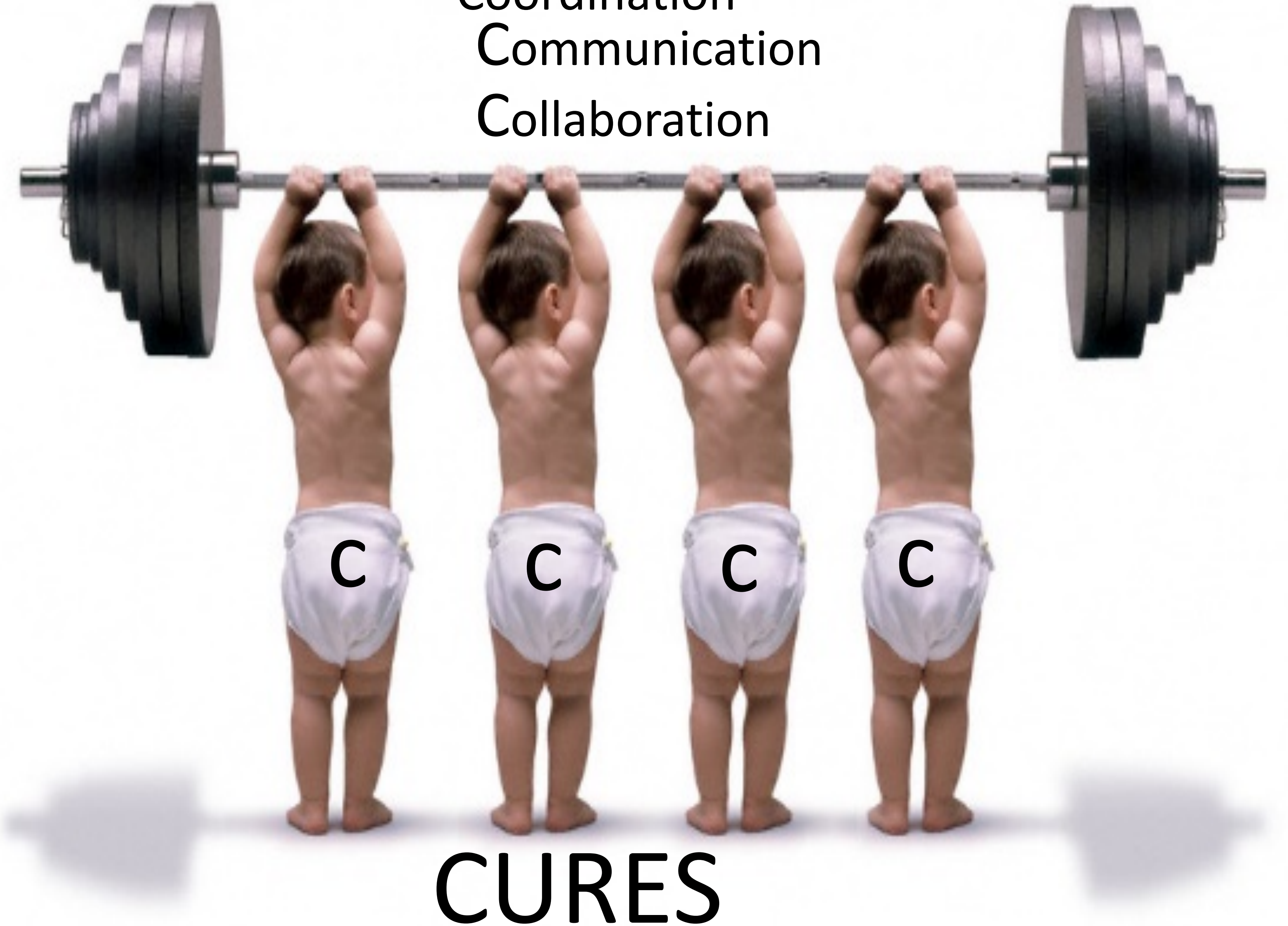
DETERMINATION

Coordination
Communication
Collaboration



CURES

Thanks for your attention!

Dr. Rashmi Gopal-Srivastava
Director, Extramural Research Program, ORDR
(Program Director, RDCRN)
Office of Rare Diseases Research (ORDR), NCATS, NIH
gopalr@mail.nih.gov
301-402-4336

NCATS

NIH National Center for Advancing Translational Sciences

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