

# Office of Rare Diseases Research

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# Office of Rare Diseases Research

- ***ORDR Mission:*** *To accelerate the translation of rare disease science to benefit patients*
- ***Major Programs and Initiatives:***

Rare Diseases Clinical Research Network (RDCRN) Program

Genetics And Rare Diseases (GARD) Information Center

Global Rare Diseases Patient Registry Data Repository (GRDR)

NCATS Scientific Conferences Program

Bench to Bedside Awards

NCATS Toolkit Project

# Office of Rare Diseases Research (2)



GARD Information Specialists can provide you with current, reliable, and easy to understand information about rare or genetic diseases in English or Spanish.



Search for Diseases, Organizations, News and More... GO

**Browse Diseases**  
View diseases by alphabetical order

**Find Support**  
Search for advocacy organizations

**Search GARD Glossary**  
Learn about medical and genetics terms

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← **GARD<sup>1</sup>**

<sup>1</sup><https://rarediseases.info.nih.gov/>

**About GARD**

The Genetic and Rare Diseases Information Center (GARD) is a program of the National Center for Advancing Translational Sciences (NCATS) and is funded by two parts of the National Institutes of Health (NIH): NCATS and the National Human Genome Research Institute (NHGRI). GARD 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](#).

**Find Out How GARD Information Specialists Can Help You**




Working together to find better treatments & improve the quality of life for individuals with rare diseases

The Rare Diseases Clinical Research Network (RDCRN) is an initiative of the Office of Rare Diseases Research, NCATS, is made up of 21 research groups (consortia) and a Data Management and Coordinating Center that are working together to improve availability of rare disease information, treatment, clinical studies, and general awareness for both patients and the medical community. The RDCRN provides up-to-date information for patients and assists in connecting patients with advocacy groups, expert doctors, and clinical research opportunities. [More about the RDCRN](#) >

**RDCRN<sup>2</sup>** →

<sup>2</sup><https://www.rarediseasesnetwork.org/>

**Rare Disease Information**

**Find a disease**  
Search the diseases we study, find consortium information, patient advocacy and study information.

**Publications**  
Download a list of publications by RDCRN researchers

**Research studies**  
See RDCRN rare diseases research.

**Rare disease research groups**  
Get to know the 21 different research groups and which diseases they are studying.

**Are YOU Interested in Research on Rare Diseases?**  
Stay Connected - Join the Contact Registry!  
Receive the most current information on:

- open recruitment for clinical studies of your disease
- opening of new clinical sites doing research on rare diseases
- activities from affiliated awareness and advocacy groups

and future opportunities to participate in research!  
[Register Today](#)

**For Patients and Families**

**Find Patient Advocacy Groups**  
The RDCRN Coalition of Patient Advocacy Groups (RDCRN 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**

**2016 Conference on Clinical Research for Rare Diseases**

**Training Opportunities**

**Other Rare Disease Initiatives**  
*Spotlight on Rare Diseases Newsletter*

**About Us**

**What is the RDCRN?**  
Arms of the Rare Diseases Clinical Research Network

**Contact Us**

# Office of Rare Diseases Research (3)



Come see our posters!



RDCRN  
Rashmi Gopal-Srivastava, PhD



Toolkit  
David Eckstein, PhD



GARD  
Henrietta Hyatt-Knorr &  
Janine Lewis

Therapy Development Continuum



Discovery

Understanding a disease process and identifying promising treatments is the first step in the therapy development continuum. It is important to get patients engaged in research from the beginning. Identifying patients' unmet needs and priorities will help scientists target their research projects accordingly. You will also want to "assemble and grow your field" by encouraging collaboration between scientists, government, and industry. You can also support research through efforts to increase funding and develop translational research tools.