

# Lysosomal Disease Network

 [consortiapedia.fastercures.org/consortia/lysosomal-disease-network/](http://consortiapedia.fastercures.org/consortia/lysosomal-disease-network/)

## Research Areas



### Tool Development

Resource



### Basic Research

## At a Glance

- Status: **Active Consortium**
- Year Launched: **2004**
- Initiating Organization: **NIH Rare Disease Clinical Research Network**
- Initiator Type: **Government**
- **Rare** disease
- Location: **North America**

## Abstract

The combined and integrated efforts of the Lysosomal Disease Network will focus limited resources toward creating a network of centers with expertise in one or more of these diseases in order to solve major challenges in diagnosis, disease management, and therapy. Solutions to these problems will have direct impact on patients suffering from lysosomal diseases and important implications for medical practice.

## Mission

The network is studying the following diseases:

alpha-Mannosidosis types I / II

Aspartylglucosaminuria

Batten disease

Batten disease, late infantile

beta-Mannosidosis

Cystinosis

Danon disease  
Fabry disease  
Farber disease  
Fucosidosis  
Galactosialidosis types I / II  
Gaucher disease  
GM1-Gangliosidosis types I/II/III  
GM2-Gangliosidosis  
Hunter syndrome  
Hurler syndrome  
I-cell disease  
Krabbe disease  
Maroteaux-Lamy syndrome  
Metachromatic leukodystrophy  
Morquio syndrome  
Mucopolidosis type IV  
Mucopolysaccharidosis type IX  
Multiple sulfatase deficiency  
Niemann-Pick disease  
Northern Epilepsy  
Pompe disease  
pseudo-Hurler polydystrophy  
Pycnodysostosis  
Sandhoff disease  
Sanfilippo syndrome A  
Sanfilippo syndrome B  
Sanfilippo syndrome C  
Sanfilippo syndrome D  
Scheie syndrome  
Schindler disease  
Sialidosis types I / II  
Sialuria, Salla disease  
Sly syndrome  
Tay-Sachs disease  
Vogt-Spielmeyer disease  
Wolman disease

## Structure & Governance

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This consortium is part of the Rare Diseases Clinical Research Network (RDCRN), an initiative of the Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS). The RDCRN is designed to advance medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment, and data sharing. Through the RDCRN consortia, physician scientists and their multidisciplinary teams work together with patient advocacy groups to study more than 200 rare diseases at sites across the nation.

Funding and scientific oversight for the RDCRN are provided by NCATS and 10 NIH components: Eunice Kennedy Shriver National Institute of Child Health and Human Development; National Cancer Institute; the National Heart, Lung and Blood Institute; National Institute of Allergy and Infectious Diseases; the National Institute of Arthritis and Musculoskeletal and Skin Diseases; National Institute of Dental and Craniofacial Research; National Institute of Diabetes and Digestive and Kidney Diseases; National Institute of Mental Health; National Institute of Neurological Disorders and Stroke; and Office of the Director. In addition, patient advocacy groups provide funds for many of the projects.

## Links/Social Media Feed

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Homepage <http://www.lysosomaldiseseanetwork.org/>

## Points of Contact

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