Lysosomal Disease Network

Research Areas

- Tool Development
- 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
Mucolipidosis 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

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

Homepage http://www.lysosomaldiseasenetwork.org/

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