

Search...

- Home
- -About Us
  - Participating Clinical Centers
- +Learn More
  - Disorder Definitions
  - FAQs
  - Glossary
  - Resources
- +Get Involved
  - Studies
  - RDCRN UCDC Contact Registry
  - Patient Advocacy
- +Healthcare Professionals
  - Disorder Definitions
  - Treatment Guidelines
  - Publications
  - Referring Patients To The UCDC
  - Education and Training Opportunities
- +News & Events
  - News
  - Events
- Contact Us

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## ABOUT US

Home About Us

| Login



### About UCDC

The Urea Cycle Disorders Consortium (UCDC) consists of a team of physician scientists, neuropsychologists, nurses, genetic counselors, research staff, and patient advocates throughout the US, working together to improve the lives of people with urea cycle disorders.

The UCDC is an international collaborative Consortium with sites in United States, Canada, and Europe. The sites have significant experience in the diagnosis and treatment as well as research of urea cycle disorders and are working together to improve the lives of people with urea cycle disorders.

As urea cycle disorders are rare, the number of participants that can be enrolled is limited and thus, the conduct of clinical research studies is very challenging. The Consortium provides a way for patients to join with doctors and researchers by participating in research studies. The greater the collaboration between researchers and patients, the more we can learn about these disorders. This is an important step in understanding how these diseases impact the lives of affected individuals and discover new and improved treatments.

### Mission

The UCDC is dedicated to improving the lives of individuals affected by urea cycle disorders. The mission of the UCDC is to understand the natural history of the urea cycle disorders, conduct clinical research and clinical trials to discover new treatments, form partnership with patients, patient advocacy groups, and other stakeholders and train the next generation of rare disease investigators.

We hope to accomplish our goals by:

- Continuing the enrollment of a large group of patients with urea cycle disorders into an observational study called the Longitudinal Study of Urea Cycle Disorders. In this study, subjects are followed over time and clinical, neuropsychological,

laboratory and other data are collected at regular intervals.

- Conducting clinical trials of promising new therapies for the treatment of urea cycle disorders.
- Establishing a patient contact registry.
- Discovering new laboratory markers of disease that will lead to better treatment and deeper scientific understanding of the causes of these diseases.
- Working with the National Urea Cycle Disorders Foundation to help patients who wish to be involved in research connect with doctors conducting the research.
- Constructing and maintaining an electronic website resource with updated information for clinicians, researchers, and patients.
- Mentoring and training junior investigators in UCD and rare diseases research.

## Goals

The UCDC works to achieve its mission through four major goals:

1. Further the understanding of the natural history of urea cycle disorders
2. Develop and conduct clinical research and clinical trials to discover new therapies that improve survival and outcome
3. Create resources with information on urea cycle disorders for clinicians, researchers, and patients
4. Train the next generation of rare disease investigators, particularly in the field of UCD

## UCDC Leadership

### **Andrea Gropman, MD**

Children's National Medical Center, Washington, D.C.  
Principal Investigator

### **Shawn McCandless, MD**

Rainbow Babies & Children's Hospital/Case Western Reserve University, Cleveland, OH  
Co-Principal Investigator, Administrative Director

### **Cynthia LeMons**

Executive Director, National Urea Cycle Disorders Foundation  
Co-Principal Investigator

### **Sandesh CS Nagamani, MD**

Baylor College of Medicine, Houston, TX  
Co-Principal Investigator

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