LEUKODYSTROPHY CENTER

The Leukodystrophy Center at Children's Hospital of Philadelphia provides comprehensive clinical care, diagnostic testing and the most advanced treatments available to infants, children and adolescents with inherited white matter diseases.

The Leukodystrophy Center is dedicated to providing accurate and prompt diagnosis and multidisciplinary, state-of the-art care. We pursue research that leads to clinical trials, new treatments and potential cures.

Our goal is to create standardized methods of treatment for leukodystrophy, including personalized genetic medicine, repurposing strategies, and bone marrow and stem cell transplantation. We deliver potentially curative therapies for newborns identified as being at risk for leukodystrophy through neonatal screening, as well as children diagnosed prior to the onset of symptoms.

DIAGNOSTICS PROGRAM

Our team of experts examines the clinical symptoms, MRI findings and diagnostic testing to ensure an accurate diagnosis. With clinical and research partnerships, we provide the most comprehensive diagnostic approaches available to patients, including whole genome and RNA sequencing. Research approaches are available to investigate novel genetic etiologies of leukodystrophies, and our team leads an international consortium researching unsolved leukodystrophies. Our goal is to achieve a causative diagnosis in every individual affected by leukodystrophy.

MULTIDISCIPLINARY CLINIC

Individuals living with leukodystrophy should receive care for every aspect of their condition, even if there is not always a cure. Our team is focused on maximizing our patients' function and clinical needs across a variety of systems, not just the neurologic needs of the individual. All of the providers meet with patients in the same room, on the same day, to limit recurrent trips to the hospital and improve communication between multiple specialists who can discuss the treatment plan together on the same day.

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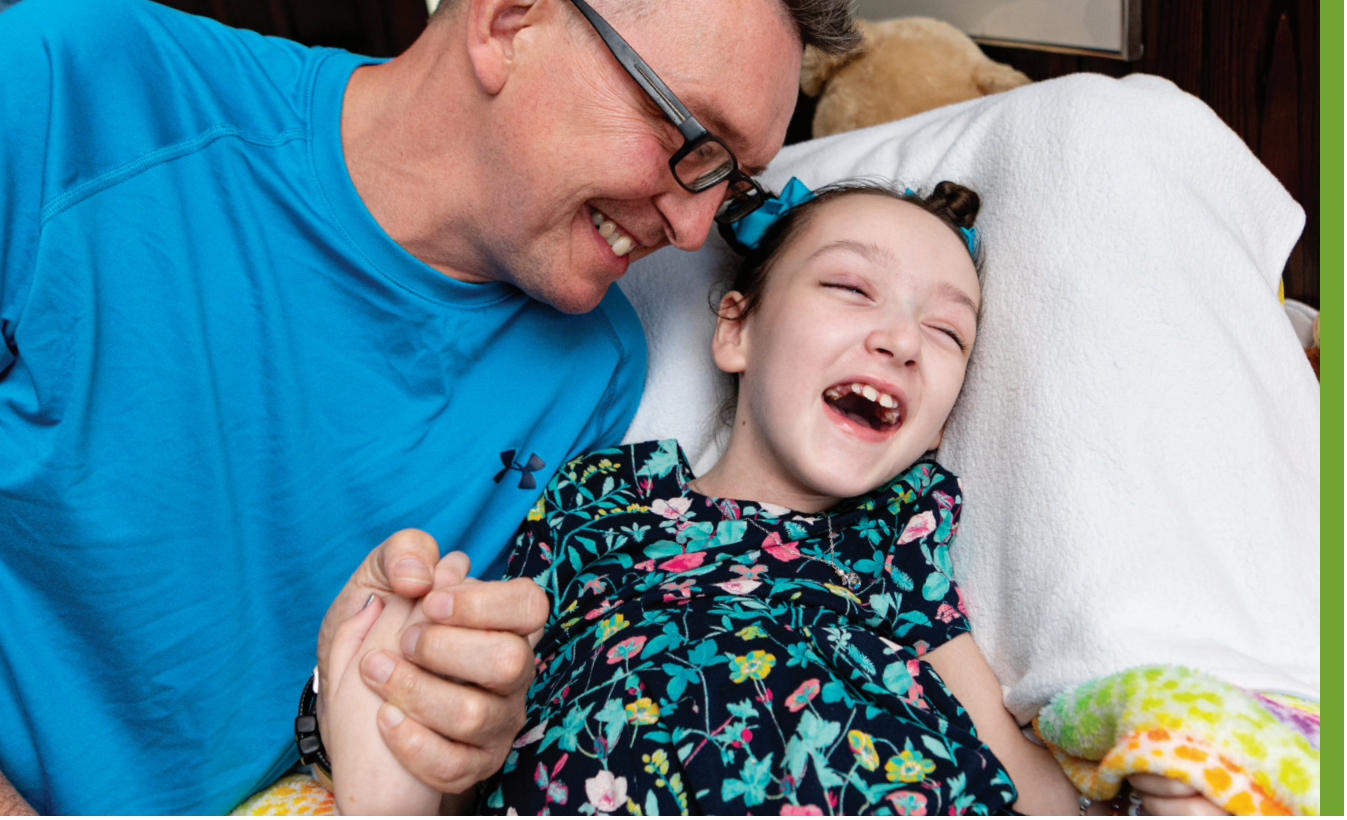
Children's Hospital of Philadelphia®
Leukodystrophy Center



Additionally, our team seeks to better understand the clinical disease progression of all leukodystrophies. The Myelin Disorders Biorepository Project contains clinical data and research samples (blood, spinal fluid, DNA) for more than 1,700 families with leukodystrophies. Our center has accumulated awards and grants to support these efforts, including two National Institutes of Health-funded studies in Aicardi-Goutieres syndrome, and the support of the NIH to participate in the Rare Diseases Clinical Research Network (RDCRN), as well as industry, philanthropy and state sponsorship of research platforms. We are proud to collaborate with numerous advocacy partners in the leukodystrophies.

We also lead a multi-stakeholder consortium, the Global Leukodystrophy Initiative (GLIA), which is funded by the RDCRN and is the first consortium focused specifically on the leukodystrophies. The key goal of the consortium is advancing treatments and clinical trial readiness.

OUR TEAM. YOUR PARTNERS.

Our interdisciplinary team includes physicians (neurologists, physiatrist, complex care pediatrician), nurse practitioners, nurses, dietitians, speech therapists, social workers, physical therapists and occupational therapists, all of whom are specially trained to care for patients with leukodystrophy disorders.

PARTNER WITH US
To refer a patient or request a second opinion:
267-426-6298
CHOPUSA@email.chop.edu

LEARN MORE
chop.edu/leukodystrophy

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