MISSISSIPPI RARE DISEASE ADVISORY COUNCIL

Rare Disease Resource List

Below you will find various resources to support patients with a rare disease, caregivers, or those seeking a diagnosis. These resources range from rare disease organizations to assistance with medical, pharmacy, travel and financial needs. This list is not exhaustive and will be updated periodically as additional resources become available or known to us.

Rare Disease Organizations

Genetic Alliance

https://geneticalliance.org

Genetic Alliance is a global organization with programs for rare disease information and advocacy.

Mississippi Metabolics Foundation

https://www.msmetabolics.org

Mississippi Metabolics Foundation offers support and resources for those living with or caring for someone with genetic metabolic disorders.

National Organization for Rare Disorders

https://rarediseases.org

The National Organization for Rare Disorders (NORD) offers a rare disease database, guides to disease diagnosis and management, and patient assistance programs.

EveryLife Foundation for Rare Diseases

https://everylifefoundation.org

The EveryLife Foundation for Rare Diseases is an organization for advocacy and advocates with a selection of useful guides and resources developed by the rare disease community.

National Bleeding Disorders Foundation

https://bleeding.org

The National Bleeding Disorders Foundation is an organization for those affected by rare bleeding disorders. They provide advocacy, support and useful resources for those affected by bleeding disorders such as Hemophilia.

Global Genes

https://www.globalgenes.org

Global Genes is a global organization for individuals and families fighting rare and genetic diseases offering support, education and advocacy.

Syngap Research Fund

https://curesyngap1.org

Syngap Research Fund is a public charity that is completely family led whose mission is to improve the quality of life of SYNGAP1 patients through the research and development of treatments, therapies and support systems.

The Bonnell Foundation

https://thebonnellfoundation.org

The Bonnell Foundation is a Cystic Fibrosis Foundation whose purpose is to provide tools to navigate the difficulties of living with Cystic Fibrosis.

The Cystic Fibrosis Foundation

https://www.cff.org/support

The Cystic Fibrosis Foundation (CFF) is a nonprofit organization dedicated to curing cystic fibrosis (CF) and helping those affected live long, fulfilling lives. It achieves this by funding groundbreaking research and drug development, supporting a nationwide network of specialized care centers, and providing tools and resources to patients and families. The Foundation also engages in advocacy to ensure access to high-quality care and essential therapies

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The Muscular Dystrophy Association

https://www.mda.org/services

The Muscular Dystrophy Association (MDA) is the leading voluntary health organization in the United States dedicated to empowering individuals living with muscular dystrophy, ALS, and over 300 other neuromuscular diseases. Since its founding in 1950, MDA has driven progress through groundbreaking research, comprehensive care, and advocacy efforts that promote independence and inclusion for affected individuals and families

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Hope for PDCD (Pyruvate Dehydrogenase Complex Deficiency)

https://www.hopeforpdcd.org

Hope For PDCD is a parent-powered nonprofit foundation dedicated to accelerating research and developing life-saving treatments for Pyruvate Dehydrogenase Complex Deficiency (PDCD), a rare mitochondrial disorder affecting carbohydrate metabolism. The foundation's mission is clear: "No cure" is not an answer. It supports cutting-edge research in genetic medicine, including AAV9 gene therapy, and promotes early diagnosis and standards of care to improve quality of life and life expectancy for affected children.

Global Liver Institute

https://globalliver.org

The Global Liver Institute (GLI) is a nonprofit organization committed to elevating liver health on the global public health agenda. Founded to address the widespread impact of liver disease, GLI promotes innovation, fosters collaboration, and scales effective solutions to improve outcomes for individuals and families affected by liver conditions

Medical, Prescription Drug and Financial Assistance

The National Organization for Rare Disorders (NORD)

https://rarediseases.org/patient-assistance-programs

The National Organization for Rare Disorders (NORD) offers a suite of RareCare® Patient Assistance Programs designed to support individuals living with rare diseases. These programs help patients:

- Access life-saving or life-sustaining medications they might not otherwise afford
- Receive financial assistance for insurance premiums and co-pays
- Obtain diagnostic testing support
- Get travel assistance for clinical trials or specialist consultations
- Access caregiver respite grants
- Receive emergency relief and educational support

The Assistance Fund

https://tafcares.org

The Assistance Fund (TAF) is an independent 501(c)(3) charitable organization that provides financial assistance to patients and families facing high out-of-pocket medical costs. TAF helps cover copayments, coinsurance, deductibles, and other health-related expenses for individuals with serious or chronic conditions. With nearly 100 disease-specific programs, TAF ensures that underinsured patients can access FDA-approved treatments without financial barriers.

BioMarin Rare Connections

https://www.biomarin-rareconnections.com

BioMarin RareConnections™ is a comprehensive patient support program designed to assist individuals and families navigating the complexities of rare genetic conditions. The program offers one-on-one financial navigation, helping patients understand insurance coverage, identify financial assistance options, and access BioMarin therapies throughout their treatment journey.

They offer financial assistance for:

- Brineura (Cerliponase alfa) – Neuronal Ceroid Lipofuscinosis type 2 (CLN2 disease)
- Kuvan (Sapropterin dihydrochloride) – Phenylketonuria (PKU)
- Naglazyme (Galsulfase) – Mucopolysaccharidosis VI (MPS VI)
- Roctavian – Hemophilia A (Gene Therapy)
- Palynziq (pegvaliase-pqpz) - Phenylketonuria (PKU)
- Vimizim (elosulfase alfa) - Mucopolysaccharidosis type IVA (MPS IVA)
- Voxzogo (Vosoritide) - Achondroplasia

Vertex GPS

https://www.trikafta.com/patient-support-program

Vertex GPS™ (Guidance & Patient Support) is a personalized support program for individuals prescribed TRIKAFTA®, a treatment for cystic fibrosis (CF). Once enrolled, patients are paired with a dedicated GPS Support Specialist who provides one-on-one guidance throughout the treatment journey.

The OneGene Program

https://www.zolgensma.com/onegene-program

The OneGene Program® is a personalized patient support service offered by Novartis for families navigating treatment with **ZOLGENSMA®** (onasemnogene abeparvovec-xioi), a one-time gene therapy for spinal muscular atrophy (SMA).

Teva Shared Solutions®

https://www.austedo.com/tardive-dyskinesia/support

Teva Shared Solutions® is the official patient support program for individuals prescribed AUSTEDO XR® (deutetrabenazine), a treatment for tardive dyskinesia and chorea associated with Huntington's disease.

Patient Transportation Resources

PALS SkyHope

https://skyhope.org

PALS SkyHope, operated by Patient AirLift Services (PALS), is a nonprofit organization that provides **free air transportation** for individuals who need to travel for medical diagnosis, treatment, or follow-up care but are unable to afford or access commercial flights. The program connects patients with volunteer pilots who donate their time and aircraft to ensure timely access to critical care.

Angel Flight

https://www.angelflight.com/

Angel Flight is a nonprofit network of volunteer pilots and organizations that provides free air transportation for individuals in need of medical treatment far from home. Operating across the United States through regional affiliates like Angel Flight Central, Northeast, and West, the program connects patients with pilots who donate their time, aircraft, and fuel to ensure access to life-saving care.

Mercy Medical Angels

https://www.mercymedical.org/

Mercy Medical Angels is the largest charitable medical transportation system in the U.S., providing free, non-emergency transportation for patients who must travel long distances to access critical medical care.

Children’s Flight of Hope (CFOH)

https://childrensflightofhope.org/

Children’s Flight of Hope (CFOH) is a nonprofit organization that provides free air transportation for children battling rare or serious medical conditions. Their mission is to ensure that distance and cost are never barriers to accessing life-saving care. CFOH secures commercial flights for children and one caregiver, enabling families to reach specialized medical facilities across the country as often as needed and for as long as necessary.

Patient Support Resources

Courageous Parents Network (CPN)

https://courageousparentsnetwork.org/

Courageous Parents Network (CPN) is a national nonprofit organization that orients, empowers, and supports families caring for children with serious medical conditions. Through a rich library of over 600 videos, podcasts, blog posts, and downloadable guides, CPN shares the lived experiences of families and insights from clinicians to help others navigate the emotional, medical, and practical challenges of pediatric illness.

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