

Global Liver Institute

PEDIATRIC AND RARE LIVER DISEASES PORTFOLIO



Global Liver Institute's (GLI) Pediatric and Rare Liver Diseases portfolio promotes rare disease advocacy and awareness of diseases. The Pediatric and Rare Liver Diseases Council consists of over **37** member organizations including patient advocacy groups, medical societies, and industry leaders in the rare disease field. The council brings together the voices of patients and stakeholders to increase awareness, foster innovation, and advance research, clinical care, and policy in the field of pediatric and rare liver diseases.



GLI Pediatrics and Rare Liver Diseases Vision

- Improved timely diagnosis rates of rare diseases by 2030
- Development of rare specialist centers throughout the world
- Accelerated progress in pharmaceutical research and development in rare liver diseases
- Robust patient engagement and active involvement in their own healthcare

Achievements

- Hosted an Externally-Led Patient-Focused Drug Development Meeting (EL-PFDD) on primary biliary cholangitis (PBC) featuring diverse patient perspectives
- Worked to pass legislation of the Living Donor Protection Act of 2021 in Kentucky during Rare Across America
- Conducted webinars to address the significant challenge of cholestatic pruritus in rare liver diseases
- Became members of the Global Genes RARE Foundation Alliance and presented at the Global Genes Summit and RARE Drug Development Symposium
- Moderated a panel at the BIO Clinical Trial Diversity Summit

Rare diseases affect
400 MILLION
people worldwide

NIH, 2021

“Thinking about the next generation of nonpatients, everybody has a liver and everybody has a risk of liver disease.”

– **Donna R. Cryer, JD**
founder, CEO and 29-year liver transplant survivor



2024 Goals

- **Encourage** patients, healthcare professionals, and other stakeholders to recognize transitions in care through our Rare Liver Diseases Month awareness campaign in February
- **Promote** greater collaboration on projects and initiatives in the rare liver disease space by increasing the number of patient advocacy groups and disease state groups in our council by 20%
- **Equip** patients with knowledge about their disease by expanding our database of educational resources for rare liver diseases
- **Advance** our policy agenda by collaborating with partners to promote a minimum of two legislative initiatives

The average time for a patient with a rare disease to receive an accurate diagnosis is nearly **5 YEARS**

NIH, 2014

What We Do

Educate through

- Educational webinars
- Comprehensive resources

Build relationships through

- Patient advisory boards
- Partnerships with disease-specific organizations
- Fostering international collaborations

Champion advocacy efforts in

- Newborn screening
- ICD-10 coding
- Transplantation
- Orphan drugs



#RAREAWARE

2023 #RareAware at a Glance

The annual #RareAware campaign aims to raise awareness and education about rare liver diseases. Under the theme of Pregnancy and Parenthood, the month-long, multifaceted, interactive campaign included robust digital communication, roundtables, educational resources and more.

The campaign focused on improving the patient experience, removing barriers to access and care, and expanding on the need for more patient engagement in research protocols.

~425,000
SOCIAL MEDIA IMPRESSIONS

13
HIGHLIGHTED RARE LIVER DISEASE STATES

4
EPISODES OF GLI LIVE

3
ROUNDTABLES

3
NEW PATIENT RESOURCES



Learn more

Council Members

- American College of Gastroenterology (ACG)
- American Gastroenterology Association (AGA)
- Autoimmune Hepatology Association (AIHA)
- American Association of Autoimmune Related Disorders (AARDA)
- American Association for the Study of Liver Diseases (AASLD)
- Associazione Malattie Autoimmuni Del Fegato (AMAF MONZA ONLUS)
- Autoimmune Association
- BARE Inc.
- BioMarin Pharmaceuticals
- Center for Autoimmune Liver Diseases-University of Milano-Bicocca
- Children's PBC Foundation
- Community Liver Alliance (CLA)
- Everylife Foundation
- Foundation of the National Lipid Association
- Gilead Sciences
- Global Genes
- GlaxoSmithKline (GSK)
- Hepatitis Hilfe Österreich – Plattform Gesunde Leber
- Hospital Central de Maputo
- ICP Care
- Intercept Pharmaceuticals
- Ipsen Pharmaceuticals
- Liver Mommas & Families
- Mirum Pharmaceuticals
- NASH KNOWledge
- North American Society For Pediatric Gastroenterology, Hepatology & Nutrition (NASPGHAN)
- Preeclampsia Foundation
- PBC Foundation
- PFIC Network
- PSC Children's Hospital
- PSC Partners Seeking a Cure
- PSC Partners Seeking a Cure Canada
- PSC Support
- Takeda Pharmaceuticals
- Texas Liver Foundation
- The Assistance Fund (TAF)
- Travers Therapeutics
- Wilson Disease Association



Learn more