



*“Looking beyond the liver”*

### **What is GALA?**

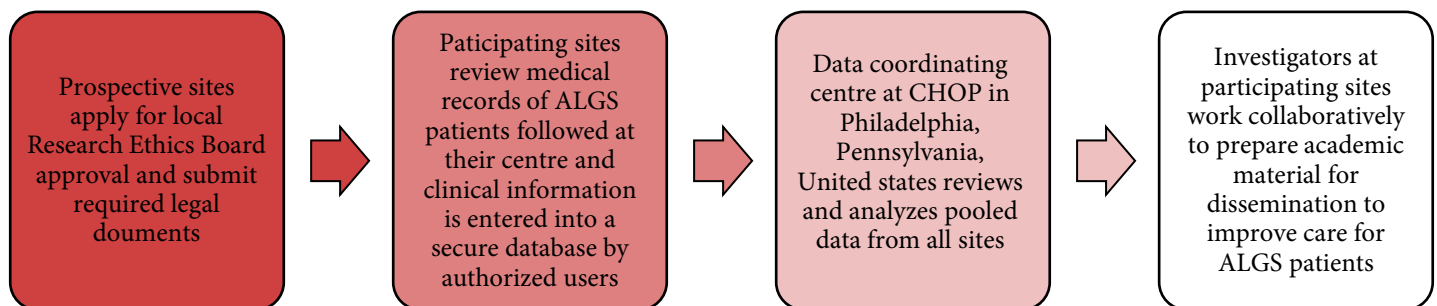
The **Global ALagille Alliance (GALA)** Study is a global initiative to create an international database of clinical, genetic, and laboratory data in children and young adults with Alagille Syndrome (ALGS). The **GALA** Study is led by **Dr. Binita M. Kamath**, a Pediatric Hepatologist and Division Chief of Gastroenterology, Hepatology & Nutrition at the Children's Hospital of Philadelphia (CHOP). Dr. Kamath is also a Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania in Philadelphia, Pennsylvania, United States. The **GALA** Study is generously supported by: The Alagille Syndrome Alliance (ALGSA) and the Lumena Pay it Forward Project (unrestricted educational grant), Mirum Pharmaceuticals (unrestricted educational grant), and Ipsen Pharma, Inc (unrestricted educational grant).

This one-of-a-kind, multi-year effort will be the bedrock for future research projects that will unravel the mysteries of ALGS, paving the way for better treatment outcomes, management, and quality of life for individuals with ALGS. To date, The **GALA** Study involves more than 100 clinicians from 34 countries around the world. The **GALA** database differs from a traditional patient registry in that only approved and verified sites may submit patient data. This is done to ensure patient anonymity, data integrity, and lawful transfer of data.

### **Why is GALA needed?**

Rare diseases like ALGS are often understudied due to limited funding and small numbers of patients in follow up at a single centre. Furthermore, current research initiatives primarily focus on ALGS patients with liver manifestations, and there is limited research in adolescents and young adults with ALGS. **GALA** aims to overcome these limitations through a comprehensive analysis of an international cohort of patients with ALGS. Doing so will allow for the determination of the prevalence, natural history, socio-economic burden, and outcomes within this population. With a focus on non-liver features, we hope to increase our understanding of ALGS and identify the unique challenges and needs of this population.

### **How does it work?**



### **What type of data is collected?**

Demographics	Clinical Characteristics	Clinical Events
<ul style="list-style-type: none"><li>• Birth history</li><li>• Genetic reports</li><li>• Family history</li></ul>	<ul style="list-style-type: none"><li>• Disease features (liver, heart, vascular, kidney)</li><li>• Laboratory/growth data</li><li>• Imaging reports</li></ul>	<ul style="list-style-type: none"><li>• Liver transplantation</li><li>• Biliary diversion</li><li>• Nutritional supports</li><li>• Pregnancies</li></ul>

### **How do I get involved?**

Those interested in participating should share The **GALA** Study research team's contact information with their healthcare team ([RABENAUQ@chop.edu](mailto:RABENAUQ@chop.edu)). For more information, please visit our website: [www.galastudy.com](http://www.galastudy.com)