



The Global ALagille Alliance (GALA) Study: Advancing Research, Changing Lives



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STUDY BACKGROUND

- The GALA Study was launched in the fall of 2017 in partnership with the Alagille Syndrome Alliance (ALGSA).
- This global effort will conduct a comprehensive analysis of an international cohort of patients with Alagille Syndrome (ALGS), a rare, multisystem, autosomal dominant, genetic disorder.
- By pooling together available datasets, the GALA Study will increase understanding of ALGS and identify the unique challenges and needs of this patient population.
- The Hospital for Sick Children and University of Toronto are leading this multi-center study.

METHODS

Study Design:

- A retrospective medical record review of children and young adults with ALGS from January 1997 to present.

Inclusion Criteria:

- All children and young adults diagnosed with ALGS from birth through 30 years old,
- ALGS defined by clinical criteria and/or *JAG1* or *NOTCH2* mutation.

Exclusion Criteria:

- None – goal is to be as inclusive as possible.

The GALA Study's first and fundamental task is to amalgamate datasets from around the world, to create a central ALGS database—which will be the bedrock of future research projects.



STUDY AIMS

The overarching aim of the GALA Study is to conduct a retrospective analysis of an international cohort of patients with ALGS to:

1. Determine the global prevalence and geographic distribution of ALGS.
2. Characterize the natural history of children with ALGS.
3. Determine the frequency and clinical spectrum of renal and vascular anomalies or events in children with ALGS.
4. Collect growth data in children with ALGS and develop an ALGS-specific growth curve.
5. Determine outcomes and causes of mortality in children and young adults with ALGS.

PARTICIPATING COUNTRIES

EUROPE

Belarus
Belgium
Bulgaria
Croatia
Czech Republic
Denmark
France
Germany
Greece
Hungary
Ireland
Italy
Poland
Portugal
Romania
Slovenia
Spain
Sweden
Switzerland
The Netherlands
United Kingdom

ASIA

China
India
Indonesia
Japan
South Korea
Taiwan



NORTH AMERICA

Canada
United States

SOUTH AMERICA

Brazil
Chile

OTHER

Australia
Israel
Jordan
Kuwait
New Zealand
Oman
Saudi Arabia
Turkey
Uganda
United Arab Emirates

NEXT STEPS

- Continue to engage and foster relationships with international collaborators.
- Begin data collection in the spring of 2018 and continue for 16 months.

STUDY CONTACT INFORMATION

Principle Investigator: Dr. Binita Kamath
Project Manager: Shannon Vandriel

Those interested in participating as a site should email gala.study@sickkids.ca for more information.

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For more information about the ALGSA contact Cindy Luxhoj at alagille@alagille.org.

