Alagille Syndrome Alliance

Stay strong. Press on.

ALGSA Overview

Mission Statement:

“Mobilizing resources, facilitating connections, promoting unity, and advocating for a cure to inspire, empower, and enrich the lives of people affected by Alagille Syndrome.”

History

- Leading 501c3 patient advocacy organization serving ALGS patients globally, established in 1997.
- 5 board members, 3 staff, an intern.
- 50+ volunteers including medical, scientific, and industry advisors in Scientific Research Network launched in 2024, and Medical Advisory Board.

International footprint

- Global leader in driving advocacy and awareness about Alagille Syndrome – A vast global network of over 7500+ individuals in our overall community of stakeholders including patients, family members, doctors, scientists and researchers.
- 15+ ALGSA-made and/or moderated support pages spanning multiple countries via Facebook and Whatsapp.
- 12+ programs that directly aid patients/caregivers/families with disease education, financial assistance, college scholarships, bereavement support, raising awareness.

ALGSA Programming

Programming dedicated to expanding access

- Targeted efforts to improve global accessibility to information, medical experts, medications, disease education.
- Support to find second medical opinions, second looks at medical records, centers of expertise, and genetic testing.
- Support letters to aid in appealing insurance denials, social security/disability denials, and for state and federal assistance.
- Provide or aid in helping find local, national, federal, and international resources.

Programming to promote education, disease awareness, essential mental health support, international families, and children in the global community. Includes:

- Age-appropriate resources for kids ex: a child and teen magazine, kids zoom sessions, and one-pagers about ALGS.
- Live zoom sessions, disease education in the form of one-pagers, manuals, guides, and videos.
- Website with touchpoints from 84 countries, international newsletter, and social media content across 3 main platforms.
- International Symposium on Alagille Syndrome in US and Simposio de Alagille en Español, a virtual symposium in Spanish.
- ALGSAbroad program – materials translated into 12 languages, includes focus groups, surveys, family highlights.

ALGSA Programming, Con't

Programming dedicated to the advancement of knowledge and science for Alagille Syndrome

- Building framework for robust Scientific Research Network to help advance understanding of how ALGS affects different organ systems.
- Cooperative Research And Development Agreement (CRADA) – Only 3-way agreement in existence in the US with NCATS/NIH, a biotech company, and a patient advocacy organization created to find the root cause of ALGS in the liver.
- Support for scientists and researchers in the field. Includes email and phone support, support letters for grants, facilitation of network connections, and much more.

In 2024, **Mental Health** is up front and center with our community members who have undergone extensive medical procedures, have long-term medical trauma, and for parents and caregivers who have carried the stress and emotional burden alongside their warriors.

2024 Priorities for ALGS community

What Matters Most to Patients?

- Responses to community surveys prior to 2021 indicate itching as the ALGS symptom that impacted QoL the most.
- In surveys following FDA approvals of Livmarli and Bylvay, itching was now ranked third, as a direct consequence of the drug approvals. **#1 – Nutritional Issues at 81%**

Additional real-life data is looking promising (Hansen et al 2023, Himes et al 2024). We expect this trend to continue.

Shift focus to IBAT intolerance and other ALGS related concerns affecting families, day to day, like:

Other Focus Areas for ALGSA

1) Nutritional issues

a. Meeting nutritional milestones that are unrealistic in the home, pressured by clinicians to reach milestones resulting in heavy emotional burden of guilt, failure, deteriorating relationships, etc.
b. GI issues in infancy with projectile vomiting, failure to thrive, malnutrition, vitamin deficiencies.

2) Skeletal involvement with ALGS

a. Bone pain persists and is lacking adequate treatment options and origin identification.
b. Fractures and dental health are catching the eye of CPS/reporters.
c. Associated hearing loss, craniofacial skeletal issues, and skull structure.

Other focus areas for ALGSA

3) Transition of Care

a. Patients and caregivers experiencing no transition plan – feeling lost in the healthcare system.
b. Coordinated care starts and stops with pediatrics leaving adults unprepared to manage coordination of medical appointments, transitioning teens, and gaslighting by clinicians and medical teams who assume they should understand and know how to take over coordination with no training.
c. Adult patients have no ALGS expert specialists or pathway to answers to health care concerns ex: reproductive health, important screenings and regular testing ex: liver cancer, kidney health, thyroid, parathyroid, regular vitamin labs, etc.

*****Adults and teens feel uncertain about their future and this feeling escalates during transition of care and for adults.**

4) Post-transplant – effects of long-term use of immunosuppressants

a. Autoimmunity/allergies: newly developing allergies, reports of anaphylaxis in community, no education about these risks pre-transplant, new dynamic of fear and trauma, no clear understanding of medical consequences of these drugs.
b. Increase in eosinophilic esophagitis.
c. Association with cancer incidences and risk.

5) Eye Phenotypes in ALGS

a. Progressive disease features and measures to mitigate them.