



ANGELMAN SYNDROME COMMUNITY ADVISORY BOARD

ASA and ASF are partnering in the ANGELMAN Community Advisory Board (CAB) which provides expert advice to all stakeholders involved in the research, development and service provision of biomedical treatments for Angelman Syndrome.

The largest worldwide Angelman Community is formed by the cooperating parent organisations in the Angelman Syndrome Alliance (International) and Angelman Syndrome Foundation (USA). Both Foundations recognize that there is a compelling need for combined efforts to advance the work that is occurring in the Angelman Syndrome pharmaceutical and biomedical research space.



Who is on the Angelman CAB?

The ANGELMAN CAB is a group of patient advocates and expert patients/carers. They utilize their professional and personal knowledge and expertise to discuss and advise on the latest developments, challenges and issues related to Angelman Syndrome.

There is an internal board of voting members when votes are required, for example if we are asked to endorse pharmaceutical and/or biomedical research. The other CAB members are there to advise and participate in crucial conversations. Each member took part in hours of training with EURORDIS to prepare them for this role. There are a total of 20 members who represent all different corners of the world including Argentina, Austria, Belgium, Canada, Czech Republic, France, Germany, Russia, Hong Kong, Hungary, Israel, Italy, Japan, Netherlands, New Zealand, Portugal, Spain, Ukraine, the United Kingdom, and the United States of America.

The ANGELMAN CAB aims are as follows

- To guide and facilitate the research and development pipeline for Angelman Syndrome treatments to better meet the needs of patients and carers.
- To increase access to, and justification for reimbursement of, Angelman Syndrome treatments globally.

- To improve awareness and understanding of Angelman Syndrome, within the healthcare system, academia, industry and institutions, and among the general public.
- To create a stronger voice for the Angelman Syndrome patient community.
- To recruit, train and support more patient advocates and experts in Angelman Syndrome to work within individual countries and across the globe as a whole.

Community Advisory Boards (CABs) are established, operated and maintained by patient advocates and expert patients. CABs are involved in scientific as well as policy-related issues in their disease area. They provide space to discuss the latest advances, challenges and issues related to medical treatments, medical devices, and procedures under development. This is an important initiative for the Angelman Syndrome community and for people affected by Angelman Syndrome globally.

What we can provide:

- Parent perspectives on living with and caring for individuals with Angelman Syndrome at all ages
- Lived experience with different manifestations of Angelman Syndrome and issues unique to specific genotypes
- Our experience with different medicine and therapies
- Guidance on how to best communicate with individuals with Angelman Syndrome
- Guidance on how to best conduct visits with AS individuals in the clinic
- Feedback on trial inclusion and exclusion criteria, informed consent forms, and retention of participants
- Assistance with evaluating the appropriateness and meaningfulness of outcome measures for individuals with Angelman Syndrome
- Insight on regulatory contexts in different countries
- Assistance to establish contact between pharmaceutical companies and clinicians/clinics
- Organizing webinars to enable communication between pharmaceutical companies and clinicians and/or the Angelman Syndrome community.
- Communication with parent association members about current and upcoming studies as requested
- Bi-directional, effective and reliable communication channel to the patients and their families.

How to set up time with the CAB?

If you would like to set up a time to meet with the CAB. Please contact:

Amanda Moore

CEO, Angelman Syndrome Foundation

amoore@angelman.org

Representing the Angelman CAB

Amanda Moore, USA

- CEO of the Angelman Syndrome Foundation
- Parent to Jackson, deletion+
- Background: Patient advocacy, collaboration

Betty Willemsen, Netherlands

- Chair of the Angelman Syndrome Alliance (ASA)
- Chair of the Nina Foundation (NF)
- Aunt to Nina, deletion +
- Background: Fundraiser, co-founder of first and largest Angelman Clinic at Erasmus MC together, City Manager of healthcare in The Hague.

Brad Schiele, Canada

- Chair of the Canadian Angelman Syndrome Society (CASS)
- Father of Ali, deletion+
- Background: Patient advocacy, strategic planning, business leadership, partnership building

Catarina Costa Duarte, Portugal

- Board member of Angel - Associação Síndrome de Angelman Portugal
- Member of the Angelman Syndrome Alliance
- Mother to Pedro, deletion+
- Background: Patient advocacy, global collaboration

Conny Schendler, Germany

- Board member of the Angelman e. V. Germany
- Member of the Angelman Syndrome Alliance
- Mother to Rike, UBE3A mutation
- Background: Patient advocacy, global collaboration

Daniel Harvey, PhD, USA

- Current member and former chair of the ASF Scientific Advisory Committee
- Former member of and current advisor to the ASF Board of Directors
- Parent to Matthew, deletion+
- Has been involved in drug discovery research in both academia and industry for more than 30 years
- Background: Vice President of Operations at Alume Biosciences where he is involved in the design and development of nerve imaging agents

Eitan Shay, Israel

- CEO of the Israeli Angelman Syndrome Foundation
- Parent to Gil, deletion+
- Background: Patient advocacy, global collaboration, establishing clinical services to Angelman patients, consulting on behalf of the Israeli community for clinical trials design and execution.

Dr. Elizabeth Jalazo, USA

- Clinic Director for the 15q Clinic Network
- Parent to Evelyn, deletion+
- Background: Pediatrician and currently a fellow at UNC in the Genetics department

Katie Cunnea, PhD, UK

- Science and Research Trustee, Angelman Syndrome UK
- Member of the Angelman syndrome Alliance
- Parent to Ruby, deletion+
- Background: Principal Scientist in Structural based drug design, Evotec

Lara Hermann, France

- President of Association Française du Syndrome d'Angelman (AFSA)
- Member of Angelman Syndrome Alliance
- Mother to Alex, Uniparental Disomy (UPD)
- Background: Patient advocacy, robust national network, cure & care perspective, support to caregivers

Nicolas Viens, France

- Board member of the Association Française du Syndrome d'Angelman (AFSA)
- Member of the Angelman Syndrome Alliance
- Father of Caroline, deletion+
- Background: Patient advocacy, national network, relationship with research teams.

Rebecca D. Burdine, PhD, USA

- Member of the Angelman Syndrome Foundation Scientific Advisory Committee and Board of Directors
- Parent to Sophie, deletion+
- Professor of Molecular Biology at Princeton University working on zebrafish models of human disorders
- Background: Patient advocacy, clinical trial steering committees, consulting for clinical trial design and execution in Angelman Syndrome, research evaluation, communication

Sabine Liermann-Campschroer, Germany

- Angelman e.V. Germany, member of workgroup science and medicine
- Member of the Angelman Syndrome Alliance
- Mother to Luis, deletion +
- Background: Patient advocacy, global collaboration, ITVs with Pharma's and researchers

