



RESEARCH GRANT 2021

The best researchers on Angelman Syndrome in the world competed for the **4th Angelman Syndrome Alliance Research Grant.**

We proudly present the recipients and their research projects:

170,000 €

Dr. Ilaria Tonazzini & Dr. Laura Baroncelli, Cnr Nano, Pisa Italy
Innovative brain-targeting nano-tools and imaging methods for therapeutic development in Angelman Syndrome (InnovAS)

The goal of the InnovAS project is to improve future therapeutic pipelines for Angelman Syndrome (AS) by;

1. Working on a less invasive method to deliver antisense oligonucleotides (ASOs) to the brain, compared to intrathecal injections. The proposed brain-delivery strategy for ASOs targeting Ube3a, will be based on biocompatible nanoparticles that can be delivered non-invasively via intranasal administration.
2. Assess non-invasive imaging methods as an unbiased biomarker for monitoring brain function in AS.

175,000 €

Dr. Simão da Rocha & Dr. Evguenia Bekman, University of Lisboa
Stem cell toolkit for modelling cerebellar dysfunction in Angelman Syndrome

This research project will investigate the hypothesis that cerebellar dysfunction significantly influences the symptoms of AS, particularly ataxia. In a stem cell-based approach, cerebellar organoids will be generated using novel induced pluripotent stem cells (iPSCs) from patients and genetically matched controls. These "cerebellum-in-a-dish" models will help future drug discovery efforts to improve the lives of patients with Angelman syndrome.

We are happy to have these strong scientists working on Angelman research. They have our faith and support!

Betty Willemsen

Chair of ASA

ASA Members

AS parent organizations from Austria, Belgium, France, Germany, Ireland, Israel, Italy, Japan, Netherlands, Portugal, Spain, Ukraine, United Kingdom and the Nina Foundation

ASA Associates

AS parent organizations from Argentina, Czech Republic, Hong Kong, Hungary

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Community Advisory Board (CAB)

Pharmaceutical companies can consult the CAB, a joint force of ASA and ASF (USA) to represent the interests of the AS community.

ASA / CAB contact

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