



Angelman Syndrome Alliance Year End Report 2020

The year 2020 was supposed to be about the Angelman trials. Multiple pharmaceutical companies were ready to start their trials with new medication for Angelman patients.

But then Covid spreaded so aggressively that some trials had to take a step back in their planning.

It was good to see that nothing was postponed for long, eventually all scheduled trials could start.

Very interesting was the start of the first global Community Advisory Board (CAB). This CAB will act as a consulting service to stakeholders involved in the research, development, reimbursement and service provision of biomedical treatments or processes, including both scientific and policy-related issues.

A lot of work has been done by the members of ASA in 2020. Always with our ultimate goal in mind, to find therapies for patients with Angelman Syndrome. We dedicate our precious time as volunteers, and we do so with love. I want to thank everybody who helps. You are like stars, you don't always see them, but you know they're there.

Thank you for your interest in Angelman Syndrome on behalf of all members of ASA.

Betty Willemsen
Chair of Angelman Syndrome Alliance

ASA Members

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

ASA Associates

AS parent organizations from Argentina, Hong Kong, Hungary

ASA Board

Betty Willemsen, Chair of ASA
Peter Sel, Vice-Chair of ASA
Manuel Trocado Costa Duarte, Treasurer of ASA

Scientific Advisory Board (SAB)

Dr. Hanoch Kaphzan
University of Haifa
Dr. Martin Scheffner
University Konstanz
Dr. Harald Sitte
University of Vienna

SAB contact

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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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Scientists funded by ASA

Ben Distel
Ype Elgersma
Ugo Mayor
Ben Philpot
Silvia Russo
Geeske van Woerden

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The Angelman Syndrome Alliance

The Angelman Syndrome Alliance is a partnership of organizations from around the globe that are focused on supporting scientific progress that benefit people with Angelman Syndrome (AS). Each ASA partner contributes with the available funds and we work as equals, supporting each other, being all part of the community of family members of people with AS.

Mission of ASA

Our mission is to grow and support scientific knowledge about AS that can create fundamental and lasting changes in therapy for people with Angelman Syndrome; so that parents, therapists and clinicians can improve the quality of life of those with AS. Together we identify research that challenges our knowledge of AS today, to enable us to develop solutions for future therapies. The alliance focuses its investments on innovative science, with our ultimate goal to find therapies for patients with Angelman Syndrome.

ASA Members

In 2020 the following countries were represented within ASA as a founding member (country & organization name in alphabetical order):

ASA MEMBERS & ASA ASSOCIATES
Argentina - Angelman Argentina
Austria - Verein zur Erforschung des Angelman Syndroms
Belgium - Angelman Syndrome Belgium
France - AFSA
Germany - Angelman e.V.
Hong Kong - HKASF
Hungary - Magyar Angelman Szindróma Alapítvány
Ireland - Angelman Syndrome Ireland
Israel - Israeli Angelman Syndrome Foundation
Italy - ORSA
Japan - The Angel Society
Netherlands - Nina Foundation
Netherlands - Angelman Syndroom Nederland
Portugal - Angel
Spain- Asociación del Síndrome Angelman
United Kingdom – Angelman UK

ASA Structure

The Angelman Syndrome Alliance has the following four corporate bodies;

- the Management Board;
- the General Meeting;
- the Meeting of Full Members;
- the Scientific Advisory Board;

Management Board

The Management Board Members are appointed by the General Meeting held on at Leuven, Belgium on 22/06/2019 for a term of four years.

The 3 Board Members are;

- Betty Willemsen – Chair person (president see above)
- Peter Sel – Vice Chair person
- Manuel – Treasurer

The Scientific Advisory Board

The Scientific Advisory Board are determined by the Meeting of Full Members. The Scientific Advisory Board consisted of;

- Hanoch Kaphzan
- Martin Scheffner
- Harald Sitte

The Scientific Advisory board is led by Harald Sitte, Chair person.

The General Meeting

The General Meeting takes place every month with the members of the Management Board. The Meeting of Full Members

At least once per calendar year a Meeting of Full Members is convened. In 2020 this Meeting has been held virtually on November 29th.

The Memo

This memorandum provides for an overview of the cooperation between organizations who – by signing the annexes to the memorandum and making (any amount of) funds available for the joint funding of research projects – are participating in this alliance. The Memo contains both a general overview of the structure as well as a more detailed description of the most important elements thereof. In 2020 we have checked if the Memo is still up to date. You will find it attached to this report.

Postponing of International Conference 2020 Vienna

Unfortunately, we had to postpone the conference in Vienna due to Covid restrictions. We will set a new date in 2022.

Community Advisory Board

The International Angelman Syndrome Alliance (ASA) and the Angelman Foundation (ASF) announced a newly formed joint initiative: The Global Community Advisory Board. They will work together to represent the Angelman community as a unified voice when engaging with pharmaceutical companies looking to enter research programmes.

The CAB will act as a consulting service to stakeholders involved in the research, development, reimbursement and service provision of biomedical treatments or processes, including both scientific and policy-related issues.

Supported by Eurordis, the European organization for Rare Disorders, the Angelman CAB will be a group of trained advocates who use their knowledge and expertise to discuss and advise on the latest developments, challenges and issues related to medical treatments and procedures under development in Angelman Syndrome. Attached in this report you find the leaflet of CAB.

Taskforces

To strengthen our organization and increase our effectiveness we started several taskforces to share the responsibility of ASA.

Communication:

Our communication needs an update on different levels. We wish to improve the quality of our information to the Angelman Community. That way we can make it clear what it is that ASA does. A taskforce is created.

News:

We wish to introduce scientists, doctors and other Angelman related personalities to our community. A taskforce I created for that purpose and several interviews have been done. You find an example attached to this report. The taskforce also organized a Q & A with professor Ype Elgersma on March 30.

Virtual meeting:

To be able to meet was quite a challenge in 2020 so therefore we started with the virtual meetings. A taskforce was able to set up to meetings. This way of getting together will be kept in the future as well.

New members:

Our goal is that every Angelman Syndrome patient and their family is able to be part of a community and to has access to the most up to date knowledge and help. By growing as a community, we can anticipate to (future) needs of individuals living with Angelman Syndrome and their families. We want the Angelman Alliance to visibly grow with new members/countries. So the Angelman Community becomes stronger. A taskforce is created.

Results 2020

Looking back on the year 2020, we can state that good progress has been made in many areas as planned. In general:

The evaluation report of three scientific research projects granted with a total sum of 335.000 has been to everyone's satisfaction;

- Prof. Ugo Mayor
- Prof. Ype Elgersma
- Prof. Ben Philpot.
- ✓ Strengthening the organization of the alliance with taskforces.
- ✓ Virtual meetings have been organized.
- ✓ Keeping overhead costs below 1 percent of revenue.
- ✓ We have excellent control over accounting and financial reporting.
- ✓ Preparing to start the next scientific research call.
- ✓ The alliance only works with (unpaid) volunteers. This includes general board members, scientific advisory board and committee members.

Finances

The Angelman Syndrome Alliance cannot achieve a thing without the moral and financial support we receive from our members. We know that some donors have to impose restrictions on the money they give us. That is why we say, we appreciate every contribution. Our special thanks to everyone who supported the Angelman Syndrome Alliance with their time and / or money.

We continue to meet our goal of devoting nearly 100% of revenue to our goal. This makes us one of the few exceptions in the world of fundraising resp. spending.

Financial Balans

ASA - Angelman Syndrome Alliance

INDIVIDUAL STATEMENT OF RESULTS BY NATURE

From January to December

Values in EURO

HEADINGS	NOTES	FISCAL YEAR	
		2020	2019
Profit and Loss			
Revenues			
Grants			
Members contributions		- €	275 000,00
Other Contributions			
Expenses			
Grants			
Researchers payments		(43 750,00)	(93 750,00)
Other expenses			
EBITDA		(43 750,00)	181 250,00
Depreciation and amortization expenses / reversals			
Impairment of depreciable / amortizable investments (losses / reversals)			
Operating result (before financing expenses and taxes)		(43 750,00)	181 250,00
Interest and similar income obtained			
Interest and similar expenses incurred		(198,77)	(41,30)
Income before taxes		(43 948,77)	181 208,70
Tax over the period's income			
Net income for the period		(43 948,77)	181 208,70

Revenues

There were no revenues in 2020. Although the research grant of 2019 has payments due in 2020, 2021 and 2022 the full amount of revenues was collected in 2019 when the grant was launched. During 2020 no other contributions were received by ASA.

Expenses

The research grant of 2019 was awarded to 3 different research labs for a total amount of € 335.000.

According to the protocols signed with the institutions only a part of this value was to be paid in 2020, and the remaining amounts will be paid in 2021 and 2022.

During 2020 the following amounts were transferred from the ASA bank account to the researchers:

- Professor Ugo Mayor - € 43.750

The bank expenses were the only other expense ASA had in 2019 in a total amount of €198,77.

Net Income for the period

The net income for 2020 was (-43 948,77€).

These results will transit to 2021 where ASA will continue to honor the commitments from the 2019 research grant (€ 137.500 to be paid in 2021 and 2022).