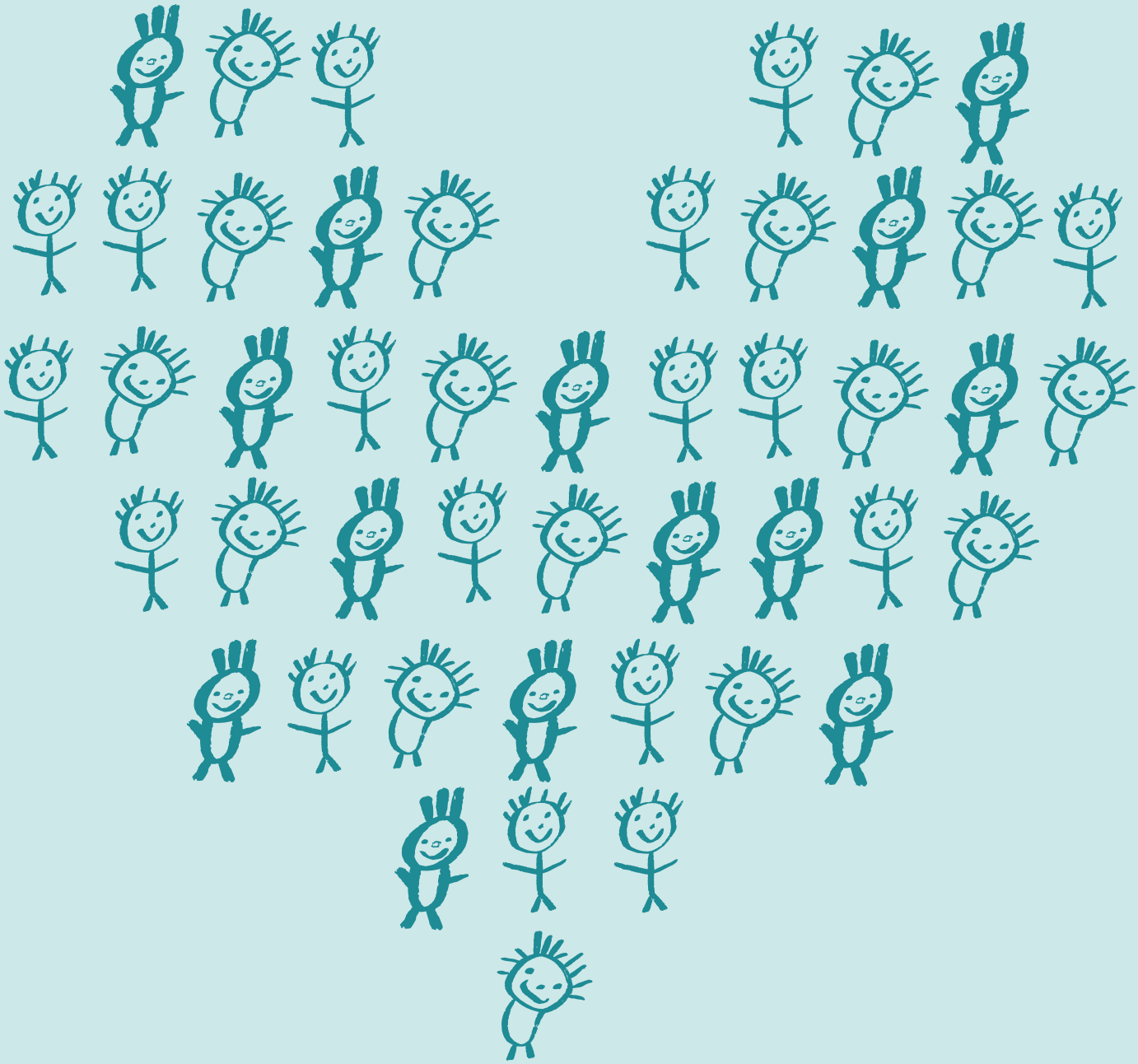
AGO ALLIANCE IMPACT REPORT 2024

AGO

In This Report

AGO



Message From The President

In 2024, we strengthened our community and foundation towards a treatment by

- Meeting for the second AGO Syndromes **Conference** (pages 11-15),
- Hearing first results of the ongoing **Natural History Study**,
- Releasing a guide for parents to understand **genetic reports** (page 19).

And something else made 2024 extra special - we **celebrated the Nobel Prize of Argonaute (AGO) researcher Victor Ambros** (pages 16-17). Delve into our annual report and listen to his videos to hear what impression our community made an him.

We extend our heartfelt gratitude to all supporters and donors for their solidarity and trust. With this generous support, we are able to undertake essential projects that unite families affected by AGO syndromes, expedite therapeutic development, and provide education.

With gratitude,
Co-founder and President of AGO Alliance

Nora

"Never doubt that a small group of thoughtful, committed citizens can change the world. Indeed, it is the only thing that ever has."
- Margaret Mead

Who We Are

Our mission

Improve the lives of children and families impacted by AGO syndromes (AGO1 & AGO2 genes) by

1. finding and funding paths to treatment,
2. empowering AGO families,
3. increasing awareness.

About us

AGO Alliance is a patient advocacy organization run by parents.
Originally established in Switzerland in 2021 with a focus on AGO2, it was re-branded in 2023 as AGO Alliance to cover further AGO genes. Sister organisations have been founded in Spain and Poland.

As a united community we access resources and create transformative change for children with AGO syndromes, surpassing the impact of individual efforts alone.

Team

Nora
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Christoph
Vice-President

Silvio
Treasurer

Aldona
Science Officer &
Parent Lead Poland

Naomi
Parent Lead USA

Antje
Parent Lead Germany

Ana
Parent Lead Spain

Ivan
Web & Graphic Support

Stefan
Design & Website

Aga
Communication

Advisory board

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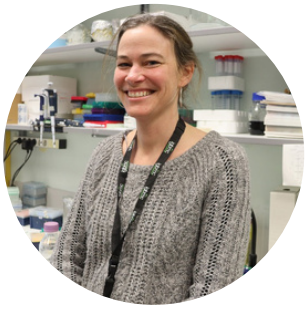
University Hospital
Salzburg

Prof. H.J. Kreienkamp
University Medical Center
Hamburg-Eppendorf

Prof. G. Meister
University of
Regensburg



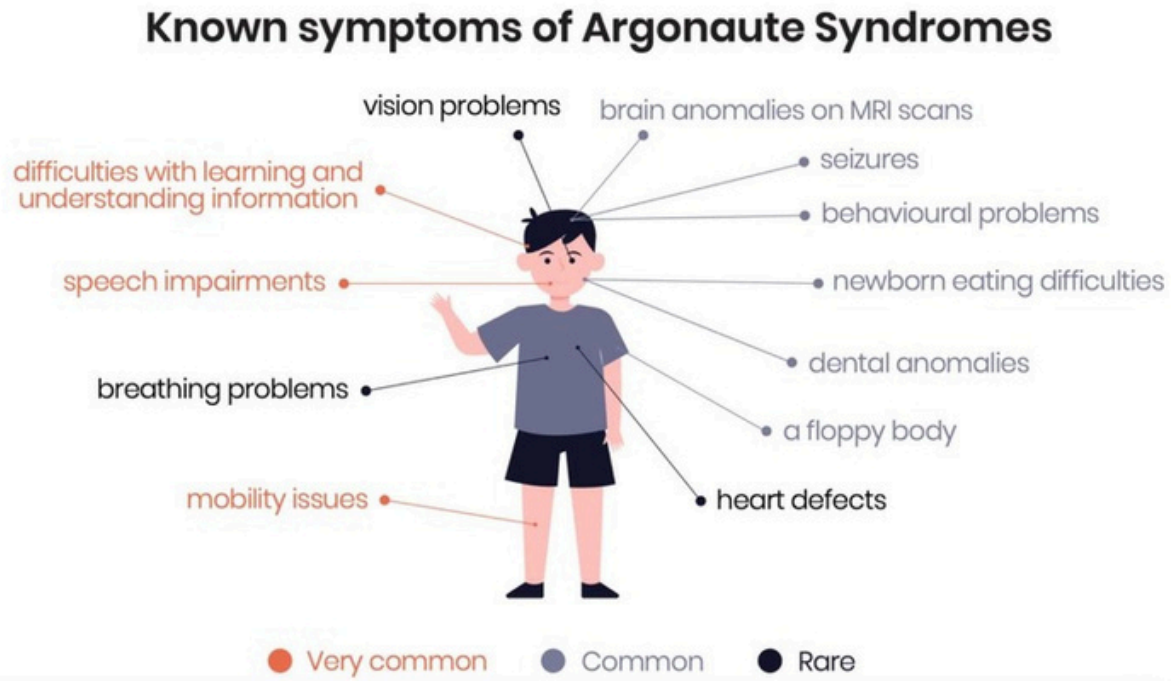
Prof. A. Piton
University Hospital
Strasbourg

What are AGO syndromes?

>130 people diagnosed with AGO syndromes worldwide

Changes to the AGO1 and AGO2 genes cause rare conditions with similar symptoms, AGO1-related syndrome and Lessel-Kreienkamp/LesKres syndrome respectively, that we collectively refer to as AGO syndromes.

AGO syndromes are extremely rare. However, as these syndromes have only been recently discovered, as many as 1 in 16,000 children may be affected.



Lessel et al., Nature Comm 2020; Schalk et al., Journal Med Genet, 2021; Lopez-Rivera et al., Brain 2020

130+ known patients with AGO1 & AGO2 mutations

55 AGO1 and 75 AGO2

Netherlands
6+

Germany
11+

USA
40+

France
5+

Spain
8+

Call to Action

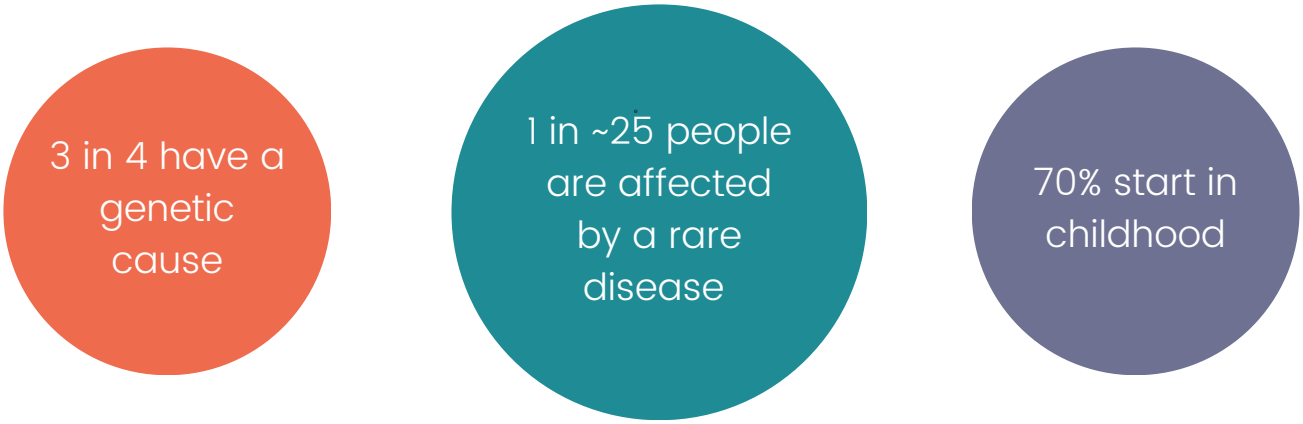
To be counted

- Join our parent group
- Send your child's mutation or genetic report and your doctor's email to piton@igbmc.fr (AGO1) or davor.lessel@ur.de (AGO2)

What is a rare disease?

Key facts

- In Europe, a disease is classified as rare when it affects fewer than 1 in 2,000 individuals.
- There are over 10,000 rare diseases identified.
- Only approximately 5-10% of these have at least one approved treatment available.



Consequently...

Research, early diagnosis, and suitable care present significant challenges, and a lack of awareness can result in misunderstanding and a sense of isolation.

> **"Only about 10% of rare diseases have an FDA-approved therapy. There is an urgent need for more research, and earlier and more accurate diagnoses of and interventions for these disorders"**
>
> - Anne Pariser, former Director of NIH NCATS Office of Rare Diseases Research

Sources: bag.admin.ch;
Wakap et al. European Journal of Human Genetics 2020
Haendel, M. et al. How many rare diseases are there? Nat. Rev. Drug Discov. 19, 77–78 (2020)

Highlights 2024

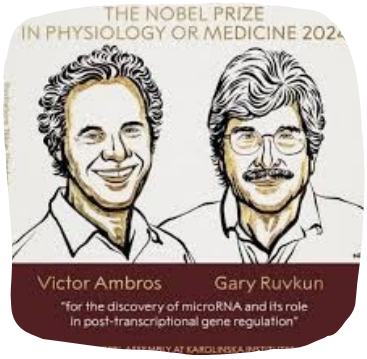
Accelerate development of treatments

Connect families

Raise awareness & educate

Organised the 2nd global AGO Syndromes Conference as a satelliet of the 2nd Argonautes meeting, in Copenhagen, Denmark

Celebrated Prof. Victor Ambros' Nobel Prize in Medicine. AGO family participated in the Nobel portrait video



Facebook support group grew to >150 members

First Natural History Study results were presented by Prof. Lessel's group at the conference

Epilepsy deep-dive kicked off by Prof. Piton

Published a guide to help parents understand genetic reports in collaboration with a student from UPenn

Science officer Aldona founds sister organisation in Poland

AGO ALLIANCE POLAND

Families across the globe received Christmas gifts

www.argonautes.ngo
>7'000 users from 98 countries since Jan 2023

AGO Syndromes Conference 2024

The second conference on AGO1- and AGO2-related syndromes took place in Copenhagen, Denmark, in August 2024 as a satellite of the second science Meeting on Argonaute Proteins.

The event successfully united scientists, industry representatives, and 40% of known patient families. It increased understanding of symptoms, shone a light on real-life challenges and encouraged new research ideas.

- *NEW* 22 patient posters were presented
- ~40 researchers attended the parent panel to learn from the experiences of patient families, and many more interacted with families during the poster session and coffee breaks

We would like to thank

- Iben, Aldona, Ivan and Antje for helping to organise the conference,
- All families who traveled to join us on-site, and who submitted posters to represent their children,
- Professor Brodersen for the support and cooperation in organising the conference.
- The Pro Dimora Foundation and individual donors for financial support, and Costello Medical for pro bono support


2024
Copenhagen - August 21-24
Argonaute
Swarts, Grohmann, Bologna, Brodersen, Vaucheret, Zamore, Gainetdinov, Ameres, Lai, Duchaine, McJunkin, MacRae, Nakanishi, Steffl, Simard, Bartel,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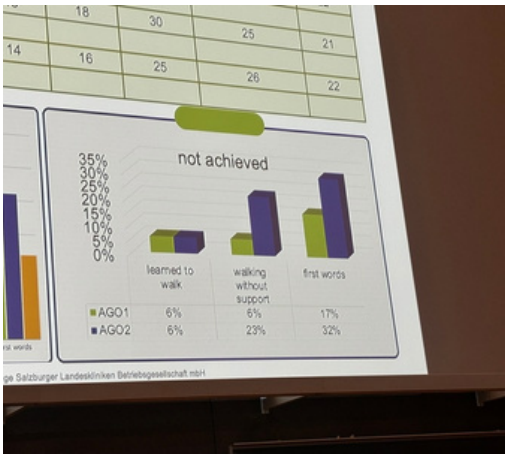

not achieved
learned to walk
walking without support
first words
AGO1 6% 6% 17%
AGO2 6% 23% 32%


JENNY
JACQUES
CARRIGAN
ALEX


JENNY
JACQUES


AGO

> This is the second time I have co-organized the science meeting and it is the best for Argonaute scientists. It truly inspires us to push boundaries and accelerate research. We're here, dedicated to working for AGO families.

Prof. Martin Simard,
CHU de Québec-Université Laval Research Center.

We felt very alone after the diagnosis, but **the association and science committee gave us hope**. This is what the conference represents for us – hope to understand the disease, to come up with a treatment, and have the **support of like-minded people** long-term. We are really grateful.

- Aldona, mum of Albert & AGO Alliance Science Officer

The best part of attending virtually was meeting other families and not feeling so alone. My daughter, Lillie, is not alone anymore. I want to do whatever I can until my last breath.

- Jessica, mum of Lillie

I have been super impressed, two years later after that first meeting with the families, at how far the field is already pushing to understand the mutations that have been identified and what they could be doing. In our own work where we look at infection models and basic biology, I genuinely think about the patients now and hope that maybe something we're learning can help them. And so, that interaction, I wish we had more of it.

- Prof. Amy H Buck, University of Edinburgh

We invite you to watch the conference recap

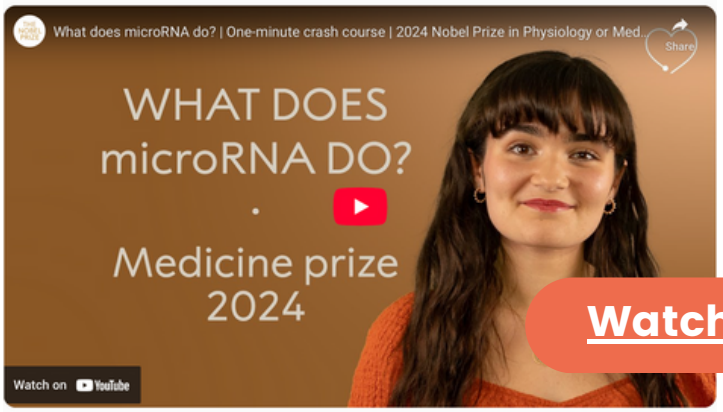
Watch video

Celebrating Victor Ambros' Nobel Prize

Argonaute researcher wins Nobel Prize

We are thrilled to congratulate Victor Ambros on being awarded the 2024 Nobel Prize in Medicine for his groundbreaking co-discovery of microRNA, which plays a fundamental role in gene regulation.

Ambros' and Ruvkun's discovery shed light on **how cells in our body become specialised**, like muscle or nerve cells. Argonaute proteins are the key partner of microRNA in this process.

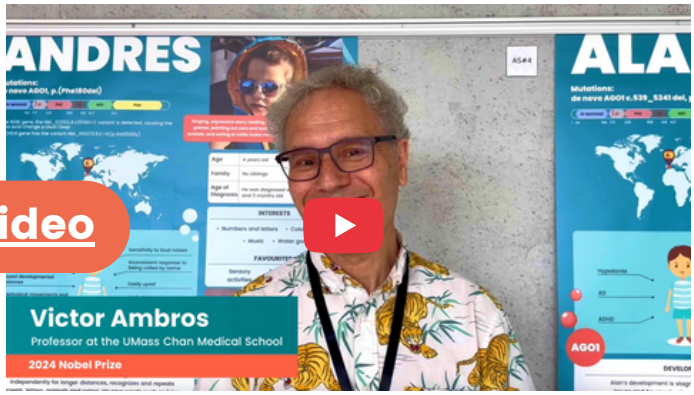


Watch 1-minute-video

At our recent conference and during the official press conference, Professor Ambros shared the powerful experience of meeting families affected by AGO Syndromes and how their stories are inspiring his and others' research. He reflected on how basic scientists are learning from patient families, advancing new insights into how Argonaute proteins work and possible therapeutic directions.

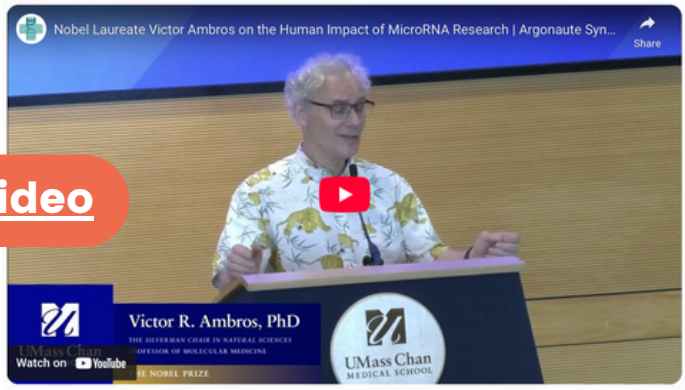
We invite you to watch his reflections during the conference and at the Nobel Prize press conference, and a portrait by Swedish television where Victor meets Daniel, a teenager affected by AGO Syndrome.

Personal reflections during the conference



Watch video

Nobel Prize Press Conference



Watch video

Nobel portrait video featuring an AGO familiy

Watch video

> **"He really sees Daniel. One of the hardest parts when you have a developmentally disabled child is the isolation. Because people don't see them for who they are."**
> Melanie, Daniel's mom, about Victor Ambros

New family resource

A Parent's Guide to Understanding Genetic Reports

A new resource is available on our website, which was created in collaboration with Hannah Sandler, a genetic counselling student from University of Pennsylvania.

This comprehensive guide is tailored specifically for families impacted by AGO gene disorders, offering clarity and support through the complexities of genetic testing reports.

Access resource

> "Collaborating with the AGO Alliance has been an absolute highlight in my journey as an up-and-coming genetic counselor. My heart is truly invested in the hope that these tools can bring comfort and clarity to those faced with the complexities of genetic testing reports."
>
> - Hannah, student at University of Pennsylvania

the orphan disease center

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AGO1/2 Natural History Study led by Prof. Lessel

Help us gain a better understanding of AGO1-/ AGO2-related syndromes!

If your child has been diagnosed anywhere in the world, please take part.

You are part of the pathway to a cure

Email

The information you provide will be used in **anonymous** form for scientific publications or presentations at scientific congresses.

Better understanding of disease is critical to developing care management guidelines, drugs and designing trials.

AGO

AGO1/2 Natural History Study

Help us gain a better understanding of epilepsy

Why?
~50% of children are affected by seizures.

This study seeks to understand the different types, and potential for diagnosis and measuring future treatment effects.

How to join
Send a physical copy of the clinical reports of consultations with child neurologists, and indicate any anti-seizure medication.

Sarah Baer/Amelie Piton
IGBMC
1 rue Laurent Fries
67 400 illkirch
France

Questions?

You are part of the pathway to a cure

Our Partners

AGO ALLIANCE POLAND

Asociación de Síndromes AGO

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Alliance Maladies Rares – Suisse
Allianz Seltener Krankheiten – Schweiz
Alleanza Malattie Rare – Svizzera

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rare village FOUNDATION

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TGE
Transnational Giving Europe

Swiss Re Foundation

Financial Report 2024
Balance Sheet

	31.12.2024 (in CHF)	31.12.2023 (in CHF)
Assets		
Liquid assets	48 329	44 045
Deferred income	0	373
Total assets	48 329	44 418
Liabilities		
Association assets	44 418	46 500
Profit/Loss	3 911	-2 082
Total liabilities	48 329	44 418

Financial Report 2024
Profit & Loss

	31.12.2024 (in CHF)	31.12.2023 (in CHF)
Revenue		
Patron contributions	11 323	0
Private donations	9 293	4 965
Other revenue	810	0
Total revenue	21 425	4 965
Expenses		
Project expenses, mainly conference	15 373	4906
Salaries, Compensation	0	714
Bank fees, currency differences	1 025	934
Memberships, Education, IT	743	494
Extraordinary expenses	373	0
Total expenses	17 515	7 048
Profit		
Profit (savings) / Loss	3 911	-2 082

We would like to thank Costello Medical, the Swiss Re Foundation and Vanilla for their pro bono support.

In 2024 the board volunteered over 200 hours.

AGO Alliance is a non-profit organisation and pursues neither commercial nor self-help purposes. Our financial books are externally examined.

Hanna-Laura Mock
Hanna_laura@hotmail.com
Neuwiesenstrasse 2, 8610 Uster

INDEPENDENT AUDITOR'S REPORT

To the general meeting of AGO, Zurich

Report on the Financial Statements

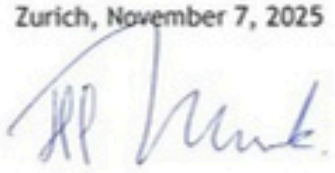
I have audited the annual financial statements of AGO for the fiscal year ended on December 31, 2024.

My responsibility is to express an opinion on these financial statements based on my audit. I examined whether the balance sheet items were supported by evidence, invoices tie to journal entries on a sample basis and the accounting records are clear and understandable.

In my opinion, the accounting records and financial statements comply with Swiss law and the company's articles of incorporation.

I recommend that the financial statements submitted to you be approved with a profit of CHF 3'911.

Zurich, November 7, 2025



Hanna-Laura Mock

We Thank You For Your Support

Acknowledgements

Our heartfelt thanks to our supporters, donors, scientific advisors, and all those who worked hard behind the scenes. In particular, Stefan Kempf for our website.

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CH-8008 Zürich

www.argonautes.ngo
contact@argonautes.ngo

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