Rare disease life means life with **QUESTIONS**

Every patient deserves **ANSWERS**

It's time to **CURE DADA2**

dada2 FOUNDATION

2023 MOMENTUM REPORT

WWW.DADA2.ORG
INFO@DADA2.ORG

TOGETHER, WE CAN FIND THE ANSWERS.

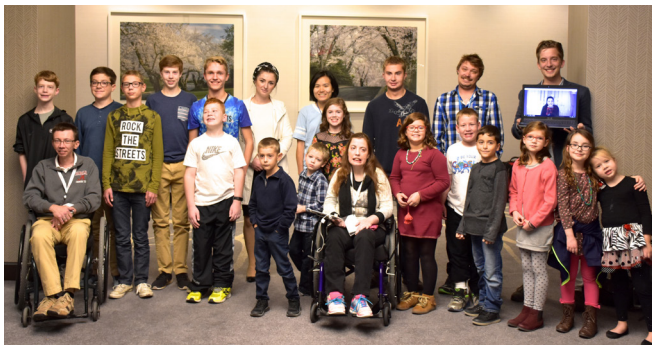
In 2014, a group at the NIH in Bethesda, Maryland, and a group in Jerusalem, Israel, described for the first time a new disease call Deficiency of Adenosine Deaminase 2, or DADA2. Since then, we have developed a global network of clinicians and scientists serving at the world's most renowned institutions, who are working hard every day to understand the disease, find treatments, and one day a cure!

Here's what we KNOW today:

- We KNOW DADA2 is genetic, appearing when a child receives one abnormal copy of the ADA2 gene from each parent.
- We KNOW these patients suffer from early onset stroke due to vasculitis, immunodeficiency, and bone marrow failure.
- We KNOW symptoms are wide-ranging and can be mild to debilitating to lethal, from rashes and fevers to life-altering infection and stroke.
- We KNOW there are medications on the market today for other illnesses that can help reduce inflammation and therefore symptoms.

But what we DON'T KNOW is what matters most:

- What does the ADA2 enzyme actually do in the human body?
- Why are the symptoms different from one patient to the next?
- Who is suffering from DADA2 but doesn't yet know it?
- What medications will work to make everyday life easier?
- And...what is a cure?



Patients at the International Scientific Conference

The time to answer all of these questions is now. According to 2021 research, nearly 35,000 people might be living with DADA2 world-wide. Yet today, we only know of +/- 600 patients. Researchers are studying enzyme replacement, bone marrow transplant, gene therapy, and other therapies that could be pathways to a cure. **But there is so much more to learn.**

The DADA2 Foundation exists to help patients close that gap - from sick and searching for answers, to treated and living the quality of life each patient deserves. We are the only organization in the world dedicated to bringing together the knowledge and expertise of more than 500 researchers and clinicians who are either doing research on or treating DADA2 patients. We also work with today's biotech and pharma leaders. And, perhaps most important, we unite patients and families around the globe. We are a daily connection, a regular convener, an always-on collaborator, and a financial supporter to these leading experts who are working with patients to find the most comprehensive, life-long cure for the disease, in the fastest time possible.

We will not stop until every DADA2 patient in the world can access the care they need - all the way up to a cure.

In our patients' words...

JEN, USA
"I wonder how close we are to a cure and how life will change for my family once it is found."

ISHAAN, INDIA
"The biggest question has to be: When could we expect to have a cure for DADA2?"

MIEKE, SOUTH AFRICA
"I don't know anyone else in South Africa with DADA2. Where can I find a community that knows what I go through?"

500+
Hosted 3 international scientific conferences in 7 years, reaching 500+ researchers and clinicians in 39+ countries

250+
Hosted 3 patient-centered gatherings in 7 years, reaching 250+ patients and their families in 20 countries

150+
Engaged with the scientific community that has published 150+ papers on DADA2

3 Helped establish ICD-10 code within first 3 years of disease discovery

Manage Family & Friends Facebook Private Group, answering questions from patients on a daily basis

In 2023, the DADA2 Foundation will be the CATALYST COLLABORATOR to engage and execute on KEY INITIATIVES that FIND ANSWERS

PUBLISH A GLOBAL CONSENSUS STATEMENT TO INCREASE AWARENESS. More than 30 clinical researchers from world-renowned institutions collaborated on a roadmap for doctors to follow when they encounter a suspected case. This clearly defined series of statements can be shared with more pediatricians, ER and rheumatology doctors, etc., who can then diagnose and treat patients with greater speed and accuracy. This paper is submitted for publication, expected in 2023.

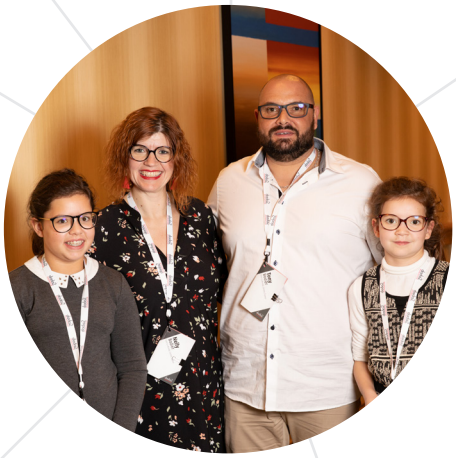
LAUNCH A PATIENT NATURAL HISTORY STUDY & PATIENT REGISTRY TO UNDERSTAND THE DISEASE. Currently in beta testing & development, this secure database will chronicle symptoms, lab tests, medications, imaging studies, medical visits - even daily life experiences - that researchers and doctors can access to study the disease. The DADA2 Patient Registry will pay in dividends, as it is the foundation from which patterns in symptoms and care can be identified, treatments can be tracked, and an eventual cure can be developed. We are about to begin recruiting all DADA2 patients and families to participate!

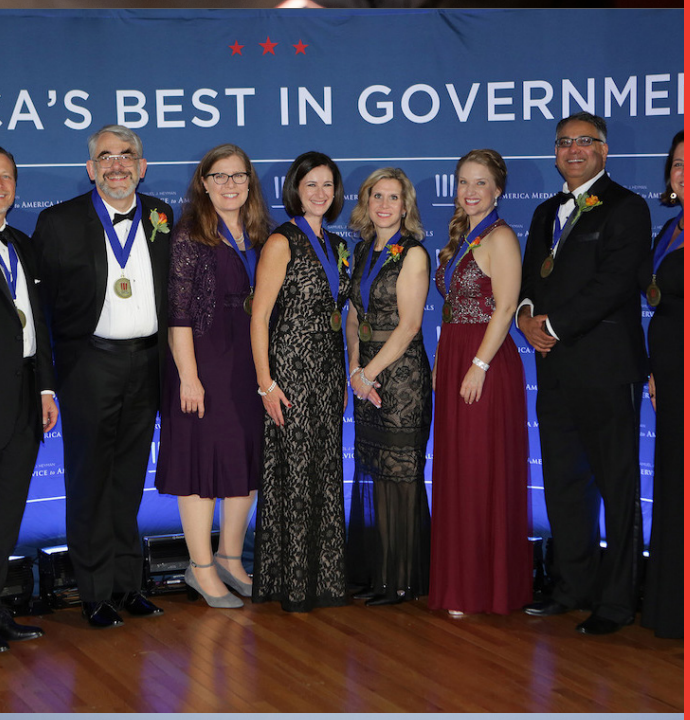
CONNECT RESEARCHERS WITH ON-THE-SPOT COLLABORATION, AND CLINICIANS WITH OTHER CLINICIANS. We will continue connecting every stakeholder involved in finding a cure through the 4th International Conference on DADA2, to be hosted October 6th, 2023, in Bethesda, Maryland. While the emphasis will be put on in-person attendance, we will also offer online attendance for those not able to travel. Further, we will launch our most comprehensive website to date, resourcing individuals with the latest in patient-focused materials and scientific literature. All of this complements our regular communications through monthly newsletters.

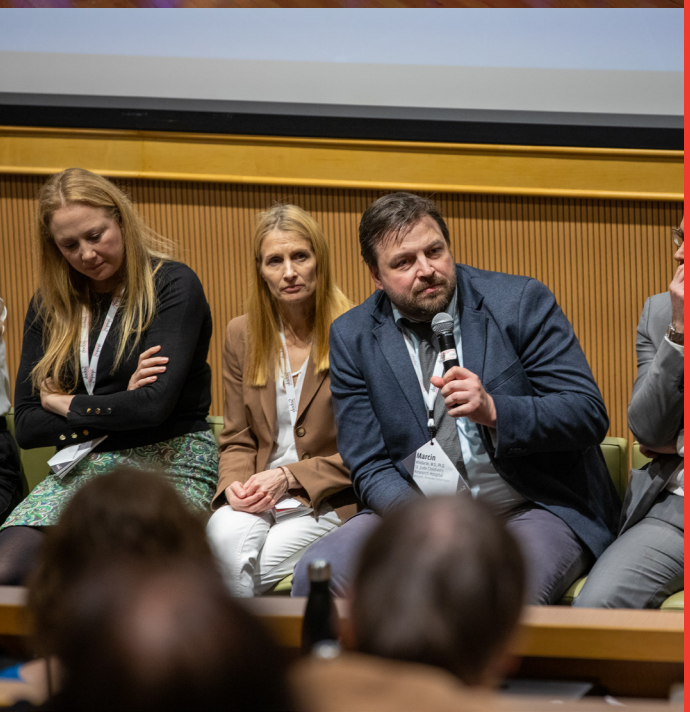
DIVE INTO MORE RESEARCH STUDIES THAT REVEAL HOW ADA2 WORKS. We will continue to explore ways to understand the mechanism of the ADA2 protein. This will help us identify new treatments. The Foundation will support these efforts by convening people, fostering collaboration among them, and supporting the work financially.

ENGAGE MORE PATIENTS.
We want to help patients find those who face the same situations, interact with their doctors in a more meaningful way, and contribute to the research agenda of the Foundation and the work it takes to find a cure.

RAISE FUNDS.
We will raise funds to support collaboration, establish tools, and ultimately fund research. We are a lean operation with a massive reach and are looking for those question askers and solution finders who want to partner with us to find a cure.







PURSUING A CURE

What is the interesting story behind the discovery of DADA2? For a long time, we didn't know what caused the many symptoms that patients were experiencing. And for decades, doctors diagnosed these patients with other known and more common diseases.

But, by 2014, researchers at the National Institutes of Health in Bethesda, Maryland, and research clinicians at Hadassah Hebrew University Medical Center in Jerusalem, Israel, were seeing similar symptoms in both their patient populations.

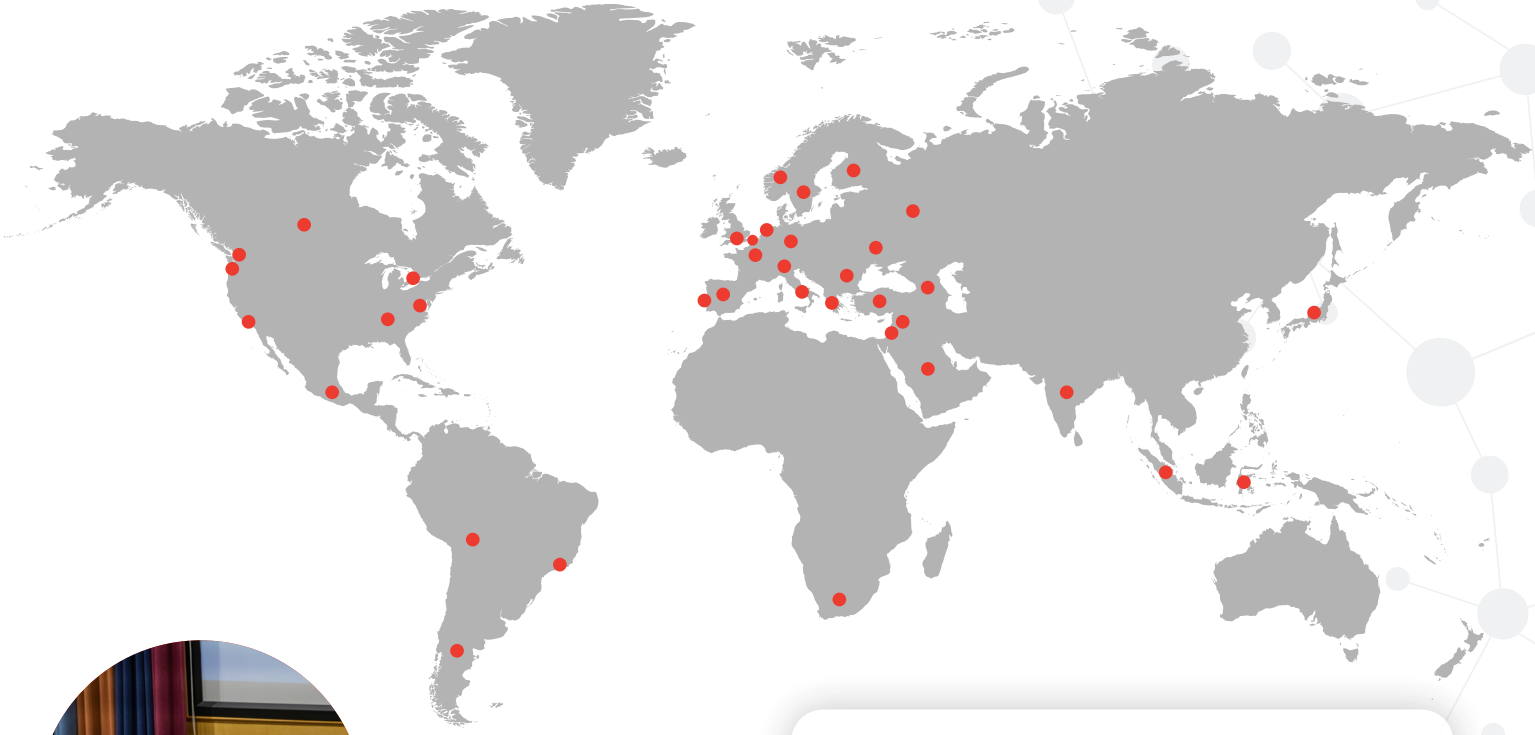
Little did they know they were discovering the same disease at the very same time!

The NIH team, led by Dr. Dan Kastner, who served as the Scientific Director of the National Human Genome Research Institute (NHGRI), was trying to treat their DADA2 patients with human plasma since the ADA2 protein is excreted into the plasma. Seventeen years ago, in Israel, a last-ditch effort to save a child led to clinicians using TNF-inhibitors, a new class of drugs that had just come on the market. It calmed the child's symptoms in 24 hours and kept them at bay.

When the two research groups serendipitously met for a completely different reason, they realized their shared work and subsequently published their findings in 2014 in the *New England Journal of Medicine* describing - for the first time in medical history - the new disease, Deficiency of Adenosine Deaminase 2 or DADA2

Right now, DADA2 patients are largely treated with TNF-inhibitor therapies, as well as symptom-reducing therapies to manage the impact of strokes. DADA2 patients with bone marrow failure generally are undergoing hematopoietic stem cell transplantation (bone marrow transplant).

GLOBAL CLINICAL AND RESEARCH ENGAGEMENT

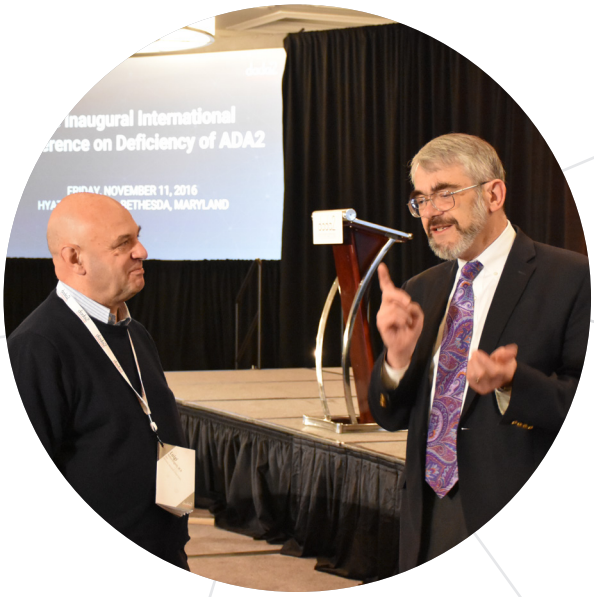


We are thrilled to partner with clinicians and researchers around the world:

- **308** conference attendees in 2020 (of 500+ registered) from 39 countries including Singapore, Greece, South Africa, Ukraine, Georgia, Indonesia, Romania, Russian Federation, Saudi Arabia, Bolivia, and more.
- **166** institutions globally.
- **129** worldwide cities.
- **39** countries.
- **180** MD; **48** MD, PhD; **45** PhD, + NPs, RNs, DOs, PharmD, Biotech leads.

Chan Zuckerberg Initiative

We are members of the inaugural group of the Chan Zuckerberg Initiative, a non-profit founded by Dr. Priscilla Chan and her husband Mark Zuckerberg, called the Rare As One Initiative – 30 rare disease organizations, all started by patients and families, receiving more than half a million dollars each to scale our organizations for future growth.



To receive the latest updates in our pursuit of a cure, find helpful resources or connect with others, visit our website and social media properties and sign up for our newsletter.

www.dada2.org

@dada2foundation (Foundation Public Page)
Families & Friends Private Group

@dada2foundation

INFO@DADA2.ORG

7051 HWY. 70 SOUTH #353
NASHVILLE, TN 37221-2207

1-615-647-8790

dada2
FOUNDATION