



# dada2

## FOUNDATION



DRIVING TOWARD CONSENSUS

August 2022

Greetings!

Time certainly does fly and the DADA2 Foundation finds itself deep in some great progress as we find a cure for this disease. As you will read in the news below, our network of clinicians is working in real-time to help patients get the right kind of care, our researchers are publishing groundbreaking findings regarding genetic research, and our patients? Well, they are taking matters into their own hands to raise money for this important work. I anticipate that, by Fall, we will also have a few very exciting initiatives to announce to this group about progress in therapeutics and in our patient registry. In the meantime, we hope just one bit of this newsletter informs your journey in caring for or managing DADA2 and inspires you along the way.



Sincerely,

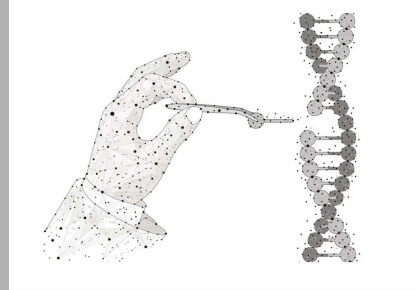
Chip Chambers, M.D.  
Founder and President, the DADA2 Foundation



Mary Makley and Julie Williams of the DADA2 Foundation and Dr. Pui Lee of Boston Children's Hospital and Harvard University, a leading researcher of DADA2, represented us this year at the [CZI Rare as One](#) gathering in San Diego in June. Take a [quick listen to the highlights of their experience](#) meeting with more than 100 rare disease patient leaders from around the country, especially the lessons and ideas gained as we pursue a patient registry and natural history study and care for a more diverse patient population as more patients are diagnosed.

## Progress Made in Gene Therapy

What is Gene Therapy? DADA2 researchers and clinicians at Great Ormond Street in London have found that gene therapy worked in a test tube and showed promise to correct the inflammation and immunodeficiency changes that DADA2 brings, and is primed for next steps in research that could lead to an eventual clinical trial. Feel free to [read the actual article here](#) and read the Q&A with the team, Dr. Despina Eleftheriou, Dr. Paul Brogan and Dr. Ying Hong.



***Give us a quick overview of how Gene Therapy could work for DADA2 patients.***

Our goal is to develop gene therapy as a safe and effective new treatment for patients with DADA2. The approach involves taking blood stem cells (immature cells that can develop into all types of blood cells) from the patient, introduce a working copy of the ADA2 gene – and then transplanting them back into the patient's body. In our recent paper we first investigated the best way of getting the gene into patient blood stem cells in the laboratory, and made sure these stem cells remained healthy and functioning after gene correction. We next confirmed that gene correction leads to reversal of the immune defects and vascular changes associated with DADA2. Our ongoing programme of research is aiming to confirm the safety and efficacy of this approach in an animal model and thereafter we will scale up our laboratory experiments before delivering this therapy to patients in the context of a clinical trial.

[Read the rest of the interview on our website here!](#)

## **Rare Disease Awareness Club**

# **BAKE SALE**

## **Tuesday 5/5-Friday 5/8**

**RARE IS MANY**  
**RARE IS STRONG**  
**RARE IS PROUD**

**in the Cafeteria during lunch**

**dada2** SHOW YOUR **SUPPORT**  
**FOUNDATION**  
For more information: [www.dada2.org](http://www.dada2.org)

## From cupcakes to treatment

Where does a bake sale at a high school in the middle of Illinois, U.S.A., fit in a newsletter full of scientific discovery? *Perfectly right here, because it's patient efforts from families like the Rickerts that lead to life-saving treatment for another family.*

Aloisia and Leontine Rickert are just in high school, and Leontine is managing DADA2 in her life. But their idea to tell their classmates about the impact of rare diseases around the world, and DADA2 in particular, led to raising

enough money to cover international flights for a family who needed a bone marrow transplant at the NIH. The group, which met regularly and eventually hosted a bake sale, raised more than \$1,300, which they chose to donate to the DADA2 Foundation.

"This is exactly the kind of thing we were hoping would result from the fundraising, since we knew the DADA2 Foundation helps families and patients access important things like medication and information," says Aloisia and Leontine.

Raising funds like this doesn't have to be complicated. Say the Rickert sisters, "We didn't really know where to start, but we were able to come up with some good ideas by sitting down and brainstorming with a group. We have a lot of help from our teacher sponsor, who had a lot of important advice and ideas."

They chose a bake sale because it didn't require any sort of prior funding to get started. Everyone involved could contribute by bringing a dessert or volunteering. They faced a few hiccups, like

exam schedules leading to skipped shifts in hosting the bake sale table. But people jumped in just at the last minute. "It felt great to see the fundraiser and everything succeeding throughout the week, even when things seemed to be going wrong."

In the past, patients have fundraised for DADA2 all over the globe - with simple efforts like Facebook fundraisers to partnering with local media outlets and hosting telethons. The opportunity really depends on what works for a family. Interested in hosting a fundraiser with DADA2? We're always happy to help brainstorm ideas. Let us know what you're thinking about at [info@dada2.org](mailto:info@dada2.org) and we'll help you out! Thanks, Rickert sisters!

**DONATE to The DADA2 Foundation**

We would love to be a part of your annual giving. Please consider a donation to the DADA2 Foundation if you are able. Otherwise, know that we are immensely grateful for your engagement!

## GET INVOLVED

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**The DADA2 Foundation**  
Pursuing a cure for DADA2, together.  
Non-profit Organization Management · Ns

### RAISE FUNDS WITH US

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### JOIN THE LINKEDIN PAGE

We've [launched a LinkedIn](#) page to keep you updated.

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