



# dada2

## FOUNDATION

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DRIVING TO CONSENSUS

December 14, 2021

## Introducing: The DADA2 Foundation's Natural History Study and Patient Registry

As we wrap up a busy 2021, I wanted to preview one of the DADA2 Foundation's top priorities for 2022. We believe that forward progress is impossible without a coordinated effort on behalf of every DADA2 patient.

That is why we are thrilled to officially share that we will be finalizing and implementing a DADA2 Natural History Study (NHS) & Patient Registry (PR)!



An NHS & PR is a large database of known patients of a particular disease or group of diseases. Using a series of survey questions, an NHS & PR collects demographic information, medical history, information about symptoms, medications, and genetic testing. Imaging and electronic health records can also be submitted.

**The DADA2 Foundation NHS & PR seeks to collect a complete medical history from every DADA2 patient in the world! Parents and siblings can also contribute. The information will be submitted by the patient, parent, or guardian, and by their physician and specialist. It will be 100% confidential!**

An NHS & PR is critical in advancing the understanding of the manifestations, symptoms, and mechanism of DADA2. It will provide researchers with a key tool in the search for better treatments and a cure.

A few key benefits include:

- Ability to estimate the prevalence
- Garner attention and interest from pharmaceutical companies
- Discover trends in the data that may suggest new treatments
- Identify key differences between patient populations
- Connect patients to researchers searching for study participants.

You don't need to do anything today. Stay tuned in 2022 for more information as we choose a platform and work with our Scientific Advisory Board to develop this exciting new

tool!

With much enthusiasm,



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

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*"Natural history studies are the bedrock upon which successful therapeutic trials are built. Natural history studies define what parameters should be observed in treatment studies, and they tell us how many subjects would be needed to see a desired effect in a clinical trial. Patient registries supercharge the process by facilitating outreach to the right number of the right patients."*

**Dan Kastner, M.D., Ph.D.**  
Scientific Director, National Human Genome Research Institute, NIH  
Bethesda, Maryland

*"Case reports and cohort studies provide incremental advancements to guide the care of rare diseases. However, to truly understand the impact of a rare disease like DADA2, a concerted effort to establish a Natural History Study and Patient Registry and collect data from patients around the world is absolutely essential."*

**Pui Lee, M.D., Ph.D.**  
Boston Children's Hospital, Harvard Medical School  
Boston, Massachusetts

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## What Patients Need to Know...

*Launching a Natural History Study (NHS) and Patient Registry (PR) will take the help of every known DADA2 patient in the world. But, we understand you may have questions! Here are a few answers.*

### Who is eligible to submit data to the Patient Registry?

- Anyone who has been diagnosed with DADA2 confirmed through a genetic test and/or an ADA2 enzyme level. For those patients under 18 years old, a parent or guardian can enter the information. Once the patient turns 18 years old, they will take over control of their data.
- Anyone who has been confirmed as a carrier of DADA2 (heterozygous mutation) through a genetic test and/or an ADA2 enzyme level.

### How do I enroll and add information?

After you have been confirmed as eligible for the NHS & PR, you will gain access to a series of surveys to be filled out via mobile or web-based platform. Individuals with DADA2, their families, and physicians will be able to enter information. Each participant will be assigned a global unique identifier, unique to that participant, to track enrollment and prevent data duplication.

### Can any doctor access the information?

No, only those approved by the Foundation. The Foundation will create strict guidelines for anyone who wants to access the data and how the data can be used. All requests to access data will be reviewed and approved by the DADA2 Foundation Governance Committee.

### Is it secure?

Data security is of utmost importance. We will choose a platform provider with a proven track record for maintaining high-security protocols!

### Who owns my information as a patient?

You are always in control of your data. By participating in the DADA2 NHS & PR, you give the DADA2 Foundation permission to share your data with approved clinicians and researchers. You can choose if your data can be shared with or without identifying details.

At any time, you can choose to withdraw your data from the registry.

### What if English is not my primary language?

DADA2 knows no geographical bounds. Our goal is to have the survey questions available in as many languages as possible. In the first year of our launch, we will focus on fine-tuning the platform and survey questions in a limited number of languages. We will then be able to focus on translating surveys to more


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

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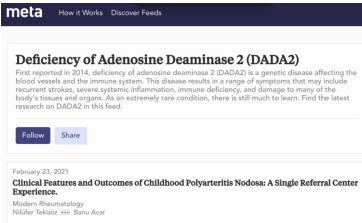
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**The DADA2 Foundation**  
Pursuing a cure for DADA2, together.  
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**Deficiency of Adenosine Deaminase 2 (DADA2)**  
First reported in 2014, deficiency of adenosine deaminase 2 (DADA2) is a genetic disease affecting the blood vessels and the immune system. This disease results in a range of symptoms that may include recurrent strokes, severe systemic inflammation, immune deficiency, and damage to many of the body's tissues and organs. As an extremely rare condition, there is still much to learn. Find the latest research on DADA2 in this feed.

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February 23, 2021  
**Clinical Features and Outcomes of Childhood Polyarteritis Nodosa: A Single Referral Center Experience.**  
Modern Rheumatology  
Niklas Tokic MD, BSc, ACer

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