



cureCADASIL

2025 Annual Report

Uniting patients, researchers, and clinicians
to find a cure for CADASIL

What You Will Find Inside



A guide to cureCADASIL's programs, research, leadership, and impact in 2025.

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Letter from the Board of Trustees

2025 was a pivotal year for cureCADASIL as we advanced our mission to find a cure for CADASIL. This Annual Report summarizes our achievements and milestones.

We welcomed new leadership to our Board: **Cheryl Gibson** of Calgary, Alberta, joined as Treasurer, and **Kathy Siegel** of Wildwood, Missouri, became our newest Board member. We also celebrated **Debra Robinson** for her five years of service as Treasurer, and **Sandra Talbird** transitioned to our Scientific Advisory Board after a decade on the Board.

Among our highlights: the third year of our **Chan-Zuckerberg Initiative** grant, our third annual **Patient-Investigator Meeting**, participation in the 12th annual **Million Dollar Bike Ride**, peer review of a record **14 grant applications**, an **\$80,000 research grant** awarded to Dr. Osama Harraz at the University of Vermont, and the launch of our **CADASIL Community Natural History Study** with NORD.

We look forward to expanding our impact in the years ahead, thanks to the support of the CADASIL community.

Sincerely,

cureCADASIL Board of Trustees



About cureCADASIL



Our Vision

A world without CADASIL.



Our Mission

cureCADASIL is a patient-led organization committed to advancing CADASIL research by uniting patients, researchers, and clinicians to find a cure.



Our Core Values

Collaboration, Community, Inclusion, Integrity, and Optimism — working on behalf of patients and families living with CADASIL.

Research is at the heart of everything we do.

cureCADASIL 2025 Year in Review



3rd

Year of the Chan-Zuckerberg Initiative Grant



2

Hosted by cureCADASIL

8

Promoted & Sponsored
(CADASIL Consortium)

10 Educational Webinars



3rd

Annual Patient-Investigator Meeting



\$65K+

Raised at 12th Annual MDBR



14

MDBR Grant Applications Reviewed
A New Record



\$80K

Research Grant Awarded to Dr. Osama Harraz

Governance and Leadership

Behind every program is a commitment to operational excellence.

1

Board of Trustees

Composed of dedicated patient and family member volunteers who bring diverse knowledge and experience.

2025 TRANSITIONS

- Cheryl Gibson joined as Treasurer
- Kathy Siegel joined as Board member
- Debra Robinson retired after five years of service
- Sandra Talbird transitioned to the SAB



2

Scientific Advisory Board

Expert researchers in epidemiology, genetics, pre-clinical studies, and regulatory pathways who guide research strategy, evaluate pharmaceutical opportunities, and review grant applications.



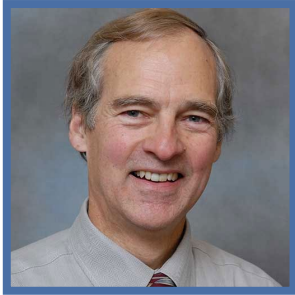
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Community Advisory Group

Patients and family members who ensure research priorities reflect the real-world needs and experiences of those living with CADASIL.



Board of Trustees



Bert Kasiske
President



Jane Gunther
Past President & Science Officer



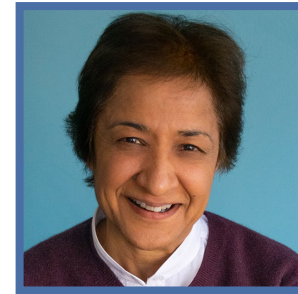
Pedro de Lencastre
Secretary



Cheryl Gibson
Treasurer



Kathy Siegel
Trustee



Vinita Bahl
Trustee

Scientific Advisory Board



Our SAB met in June 2025 under a new charter adopted by the Board of Trustees in January 2025.



Chair

Chris Hopkins, PhD, MBA



Thomas Caulfield, PhD



Hughes Chabriat MD, PhD



Clement Y. Chow, PhD



Fanny Elahi, MD, PhD



Graeme Fielder, PhD



Anne Joutel, MD, PhD



Michelle McGuinness, MS, RAC



Sandra Talbird, MSPH

The SAB guides research strategy and priorities, identifies research gaps, evaluates pharmaceutical industry opportunities, reviews grant applications, and recommends therapeutic targets for future study.

Community Advisory Group

The Community Advisory Group serve as a vital bridge between the CADASIL patient community and cureCADASIL's leadership.

Patient Bridge to Programming



In 2025, our CAG provided essential guidance to the Board and investigators by helping shape the design of the Patient Investigator Program and playing a visible role in at the Meeting (Ki Coale and June Wallach) including delivering sessions on living with CADASIL and participating in a panel discussion.

Research Leadership



CAG members ensure that research priorities reflect what matters most to people living with CADASIL, and that future clinical trials are grounded in real-world needs. The CAG also contributed to the design of our cureCADASIL Community Natural History Study.

Peer-Reviewed Publication



The CAG, cureCADASIL Board, and Elahi Lab co-authored a publication advancing patient-engaged CADASIL research titled: “Defining patient-reported outcomes and priorities for clinical trials in CADASIL through an international survey” Link: <https://doi.org/10.1016/j.cccb.2026.100534>

CADASIL Awareness Day



November 16, 2025 — the community united to raise visibility for CADASIL and connect patients and families worldwide.

CADASIL Community Natural History Study

A partnership with the National Organization for Rare Disorders (NORD)



Patient Registry: cureCADASIL partnered with NORD to design and implement a patient registry that collects real-world data from people living with CADASIL.



IAMRARE Platform: Built on the IAMRARE Platform, a secure web-based application developed by NORD for rare disease research.



Registry Transition: Replaces the former CADASIL Family Registry previously managed by Invitae, Inc. The study is owned by cureCADASIL on behalf of the CADASIL community.



Goal: Link diverse data sources about the natural history of CADASIL to facilitate research and clinical trials of potential therapies.



Open Enrollment: Open to anyone with a CADASIL diagnosis or at risk for CADASIL.

Natural History Study: Building the Foundation for Trials

Why patient registries matter for CADASIL



Connect

Create partnerships between patients, families, and investigators that encourage communication and education about CADASIL research.



Characterize

Provide a better understanding of the disease burden and rate of progression of signs and symptoms over time.



Prepare

Pave the way for therapeutic trials by helping researchers identify potential targets for treatment and design trials more likely to succeed.



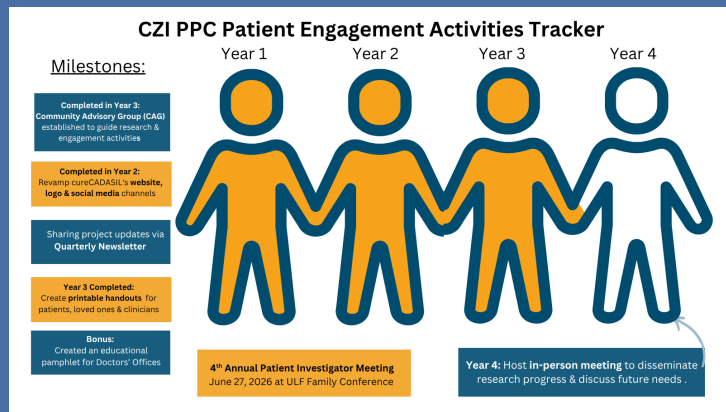
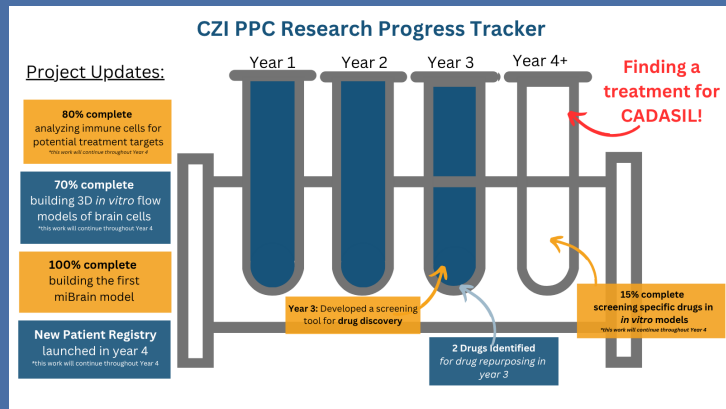
Recruit

Identify participants for upcoming research studies and clinical trials.



Data collected included: socio-demographics, medical history, diagnostics, treatment, disease progression, quality of life, clinical study participation

UPDATE: Chan-Zuckerberg Initiative



Chan-Zuckerberg Initiative (CZI)

2025 marked the third year of the CZI Patient-Partnered Collaboration, which has strengthened cureCADASIL infrastructure, supported educational programming, and enabled community events including the Patient-Investigator Meeting.

- ✓ Organizational infrastructure
- ✓ Educational programming
- ✓ Community events & meetings

CADASIL Connection – cureCADASIL Hosted Webinars



cureCADASIL Webinar Series

Immunization Therapies for CADASIL

Wednesday, June 11
11am EDT, 5pm CET

presented by
Helena Karlström
Associate Professor/
Senior Lecturer
Head of Division of
Neurogeriatrics
Karolinska Institute



Immunization Therapies for CADASIL
Helena Karlström · Karolinska Institute · June 11, 2025



cureCADASIL Webinar Series

Repurposing a Pill Therapy for the Treatment of CADASIL

Listen to the LIVE Recording

presented by
Joseph Arboleda-Velasquez
MD, PhD
Harvard Medical School,
Associate Scientist
(Schepens Eye Research
Institute)



Repurposing a Pill Therapy for CADASIL
Joseph Arboleda-Velasquez, MD PhD · Harvard Medical School

Community Resources



Empowering patients, caregivers, and providers with accessible information.



New CADASIL Handout

Released July 24, 2025, this printable resource was designed for patients, caregivers, and care providers. It includes key information to keep on hand and share when needed. Available as a free PDF download from the Resources section of curecadasil.org.



Doctor Directory

A growing directory connecting CADASIL patients with knowledgeable healthcare providers, built through years of physician outreach and education.



Support Groups

The cureCADASIL community stays connected through online resources, virtual events, and networking among CADASIL families for mutual support and advocacy.



12th Annual Million Dollar Bike Ride

June 14, 2025 · University of Pennsylvania



\$65,000+

Raised by Team CADASIL



6th Year

cureCADASIL Participated



14

A Record Number of Grant Proposals
Received in July 2025

The Penn Medicine Orphan Disease Center hosted the 12th Annual MDBR to raise funds for rare disease research. For the sixth year, Team CADASIL rallied riders, donors, and supporters to advance CADASIL research. In July 2025, a record 14 investigators submitted grant applications.

Penn Medicine Orphan Disease Center · Rare Disease Research



Research Grants and Awards

Advancing CADASIL science through targeted funding



Over **Over \$550,000** in research supported by cureCADASIL through MDBR grants between 2020–2025.

2025 MDBR Grant Award



AWARDEE

Michael Wang, MD, PhD

University of Michigan



PROJECT

Small Molecule Inhibition of NOTCH3 Conformational Alterations

AMOUNT

\$101,776

PERIOD

Feb 2025 – Jan 2027

Publication: Cartee NMP, Zhang X, Lee SJ and Wang MM. Cysteine-reactive mitigators of small vessel disease-related NOTCH3 mutants. *Sci Rep.* 2026.

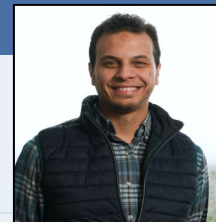
2026 MDBR Grant Award



AWARDEE

Osama Harraz, PhD

University of Vermont



PROJECT

Combating Neurovascular Degeneration in CADASIL by Targeting Piezo1

AMOUNT

\$80,000

PERIOD

Feb 2026 – Jan 2028

Research Spotlight — Innovative mechanosensitive ion channel approach to restore cerebral blood flow

Research Spotlight: Targeting Piezo1

2025 cureCADASIL Grant — Osama Harraz, PhD, University of Vermont



The Challenge

CADASIL is a rare inherited disease that damages the brain's small blood vessels, leading to strokes, memory loss, and dementia. No treatments currently exist to restore healthy blood flow in CADASIL patients.



The Approach

Our research has uncovered an overactive protein, called Piezo1, in the blood vessels of CADASIL animal models. Piezo1 appears to disrupt how brain blood flow is regulated, which may worsen symptoms. This project will test a mechanism, we hope to lay the groundwork for future therapies that could slow or prevent brain damage in people living with CADASIL and related small vessel diseases an whether adjusting Piezo1 activity can improve blood flow, brain function, and memory in CADASIL models. By targeting this newly discovered overactive protein.

\$80,000

cureCADASIL Research Grant Awarded University of Vermont

4th Annual Patient-Investigator Meeting

Saturday, June 28, 2025 · 9:00 AM – 12:30 PM CDT



VENUE

Eaglewood Resort and Spa, Itasca, Illinois 60523



PROGRAM

Presentations by patients and investigators, with questions and discussion from in-person and remote teleconference participants.



RESEARCH UPDATES

Investigators shared the latest findings on CADASIL pathology, emerging therapeutic targets, and progress in ongoing clinical studies and translational research efforts.



OPEN PANEL

An open panel discussion fostered dialogue between the cureCADASIL community and researchers.



COMMUNITY

Attendees connected at breaks and meals during the two-day ULF conference. Consensus: this annual meeting should continue and be expanded.

Major Sponsors

United Leukodystrophy Foundation

| Chan-Zuckerberg Initiative

Promoting Research Collaborations

Building bridges across institutions to accelerate CADASIL science.



Elahi–Joutel Collaboration

Announced November 11, 2025. Dr. Fanny Elahi, Director of the VascBrain Study at Mount Sinai (New York), and Dr. Anne Joutel at INSERM (Paris) formalized a collaboration to advance biomarker development and drug discovery for CADASIL.

Their partnership includes sharing biospecimens and experimental model systems to accelerate translational progress.



New York, USA

INTERNATIONAL PARTNERSHIPS DRIVING DISCOVERY



Paris, France



2025 Financial Summary

Every dollar advances our mission.

REVENUE SOURCES

- **MDBR Fundraising** **\$65K**
12th Annual Million Dollar Bike Ride
- **Chan-Zuckerberg Initiative**
Year 3 of CZI grant partnership 
- **Individual & Corporate Donations**
Community and supporter contributions 

2025 EXPENSES



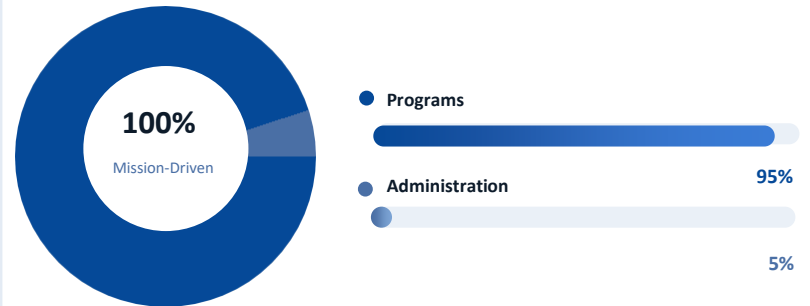
- Patient-Investigator Meeting
- Educational Webinar Programming
- Natural History Study Implementation
- Operational Costs

COMMITMENT (NOT A 2025 EXPENSE)

- \$80,000 Research Grant — Dr. Osama Harraz, UVM
Awarded as a future commitment; not reflected in 2025 spending totals.

SPENDING ALLOCATION

How funds were deployed in 2025 (excludes \$80K grant commitment)



Join the Fight Against CADASIL

Every day, individuals and families affected by CADASIL face a rare, relentless genetic disease with limited treatment options. Together, we can change that. Your support fuels the research that leads to breakthroughs. It empowers patients with resources, hope, and community. It brings us closer to earlier diagnosis, better treatments, and one day, a cure.

Your donation is not just a gift. *It is progress. It is possibility. It is hope.*



Donate

donate.curecadasil.org



Volunteer

Join our efforts



Share Your Story

Raise awareness for CADASIL

Thank You

With gratitude to everyone who made 2025 possible.

OUR PARTNERS

- National Organization for Rare Disorders (NORD)
- Chan-Zuckerberg Initiative (CZI)
- United Leukodystrophy Foundation (ULF)
- Penn Medicine Orphan Disease Center
- American Brain Coalition
- Global Genes
- National Institute of Health (NIH)
- Genetic Alliance

OUR LEADERS

- Board of Trustees
- Scientific Advisory Board
- Community Advisory Group

OUR COMMUNITY

- Donors and volunteers
- MDBR riders and fundraisers
- Webinar participants
- Patient-Investigator Meeting attendees
- Every family living with CADASIL

