

# CJD Foundation Research Grant Program



CREUTZFELDT-JAKOB DISEASE  
FOUNDATION, INC.

*Supporting Families Affected by Prion Disease*



# Research Grant Program

- Grants awarded each January to international recipients
- Topics include pathophysiology, molecular mechanisms, cell and animal disease models, diagnostics, clinical and translational research, studies identifying treatment targets or biomarkers, genetics, bioinformatics, or other areas related to human prion diseases
- Recipients present at CJDF Annual Family Conference 18 months after receiving award
- Grants can be 1 Year (up to \$50K) or 2 year (up to \$100K) and fellowships are now available

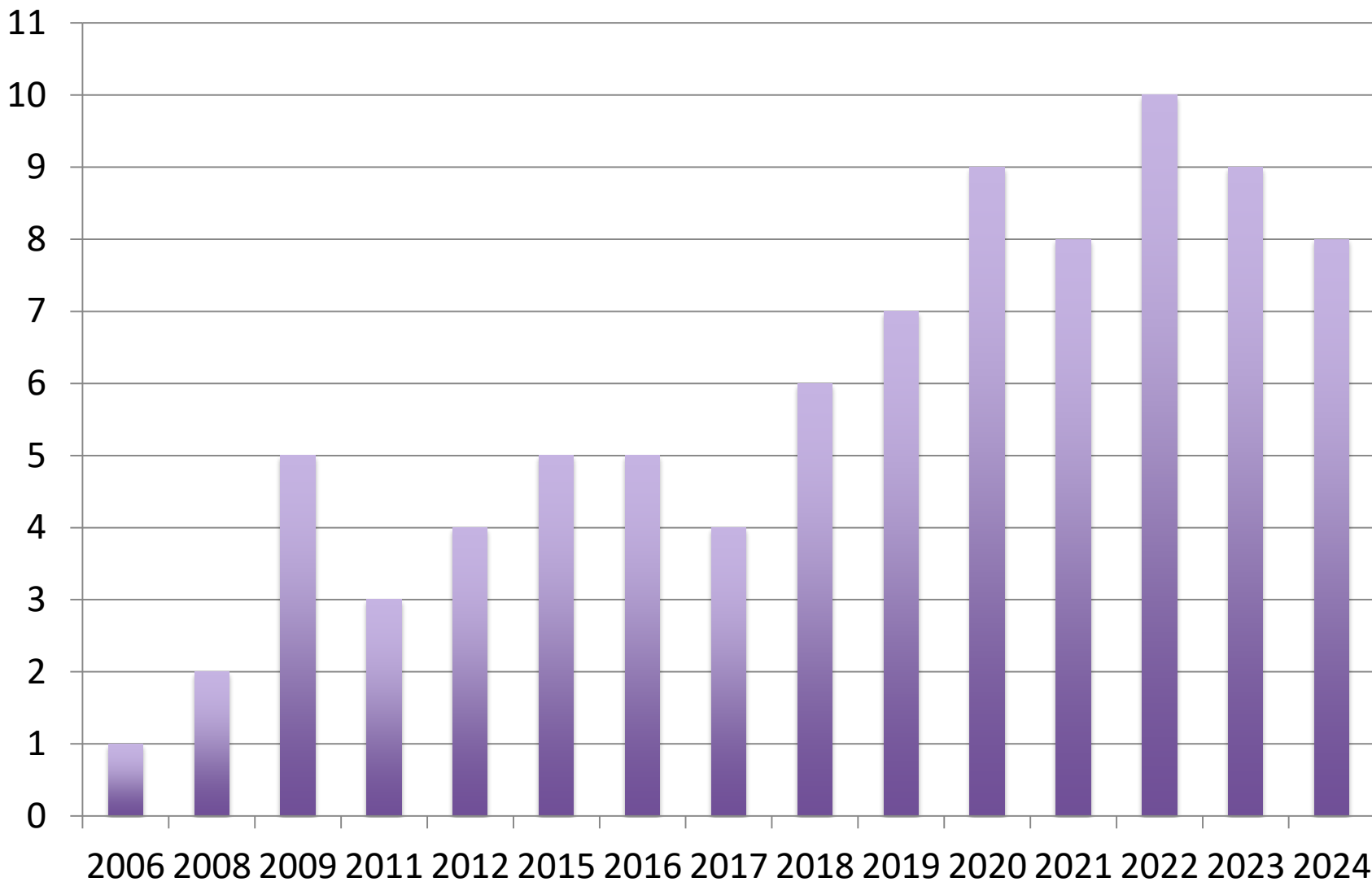


# 2024 Research Grants

- Awarded 6 grants and 2 fellowships in 2024 for a total of total 86 grants
- **2023** awardees presented reports in July 2024
- **2024** awardees will present reports in July 2025
- Funding for **2024/25** grants looks strong, thanks to Family Memorial Grants, donations to the CJD Foundation, and Strides for CJD events

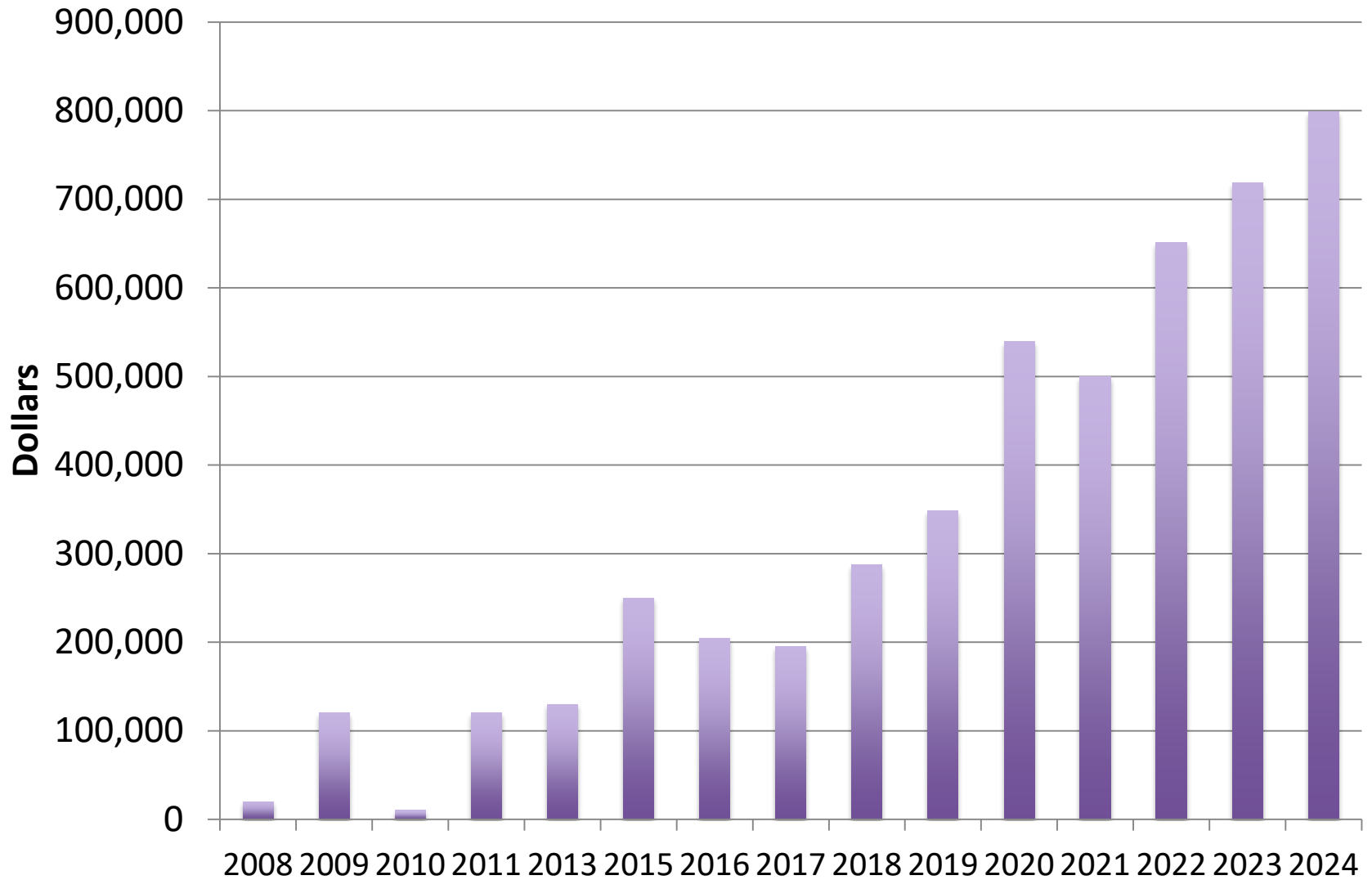


# Number of CJDF Research Grants





# Funds Awarded





# Sources of Research Grant Funding

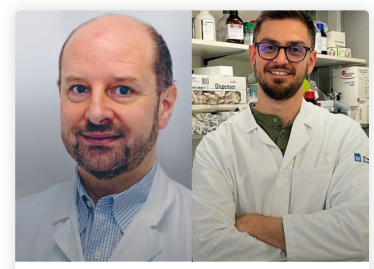
- Donations to the CJD Foundation
- Strides for CJD fundraising
- Memorial Grants
- Grant Builders / families who host fundraisers



# Research Grant Cycle

- Call for Applications from researchers: May 2024
- Applications reviewed by Scientific Advisory Committee: Summer/Fall 2024
- Donor signs grant agreement/raises funds: by September 2024
- CJDF awards grants to researchers: January 2025
- Researcher presents update at CJD Foundation Conference: July 2026

## 2024



**Dr. Adriano Aguzzi and Davide Caredio**

**Grant Title:**  
Investigation of Glycoprotein Nonmetastatic Melanoma protein B (GPNMB) as potential therapeutic target in Prion Diseases  
**Location:**

**Joaquín Castilla, PhD**

**Grant Title:**  
Detection of residual prions from decontaminated medical and laboratory surfaces  
Exploring the efficacy of dominant negative protein-based gene therapy for different prion diseases: in vivo proof-of-concept study

**Arielle Hay, Ph.D.**

**Grant Title:**  
Generating cerebral organoids from donors with sporadic Creutzfeldt-Jakob Disease  
**Location:**  
Postdoctoral Research Fellow at Rocky Mountain Laboratories, National Institutes of Health, Hamilton, Montana

**Qingzhong Kong, PhD**

**Grant Title:**  
Development of ADAM8-based Gene Therapy for CJD  
**Location:**  
NPDPSC, Case Western Reserve University  
**Grant Year:** 2024

**Dr. Surabhi Mehra**

**Grant Title:**  
Understanding the molecular mechanism of spontaneous prion emergence in knock-in mouse models  
**Location:**  
Tanz Centre for Research in Neurodegenerative Diseases, University of Toronto





# Thank You to All Who Support the Grants Program

- Donors to CJD Foundation and Strides for CJD
- Family Memorial Grant Funders
- Researchers
- Scientific Advisory Committee

# **Selected Highlights of Previously-Awarded CJD Foundation Research Grants**

**Joel Watts, PhD**

**Associate Professor**

**Canada Research Chair in Protein Misfolding Disorders  
Tanz Centre for Research in Neurodegenerative Diseases  
Department of Biochemistry, University of Toronto**

**CJD Foundation Family Conference  
July 21<sup>st</sup>, 2024**

# **Development of an Anti-Mortem and Pre-Symptomatic Diagnostic Test for vCJD and Genetic Human Prion Diseases Using Real Time QuIC**

- Grant recipient: Dr. Christina Orrú-Groveman
- Year: 2012



# Development of an Anti-Mortem and Pre-Symptomatic Diagnostic Test for vCJD and Genetic Human Prion Diseases Using Real Time QuIC



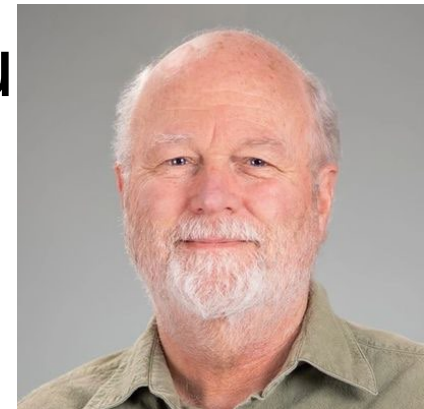
RESEARCH ARTICLE

## Bank Vole Prion Protein As an Apparently Universal Substrate for RT-QuIC-Based Detection and Discrimination of Prion Strains

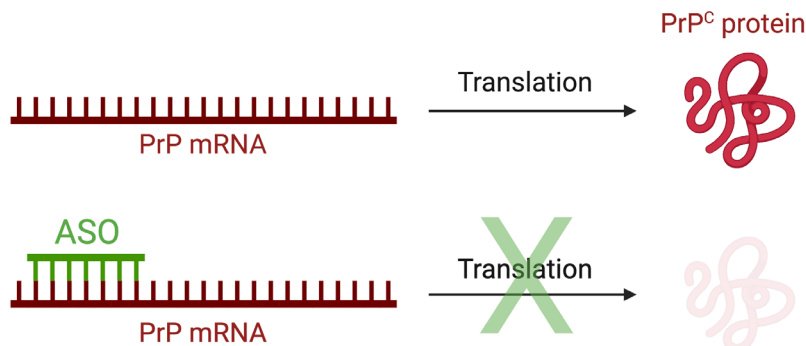
Christina D. Orrú<sup>1</sup>✉, Bradley R. Groveman<sup>1</sup>✉, Lynne D. Raymond<sup>1</sup>, Andrew G. Hughson<sup>1</sup>, Romolo Nonno<sup>2</sup>, Wenquan Zou<sup>3</sup>, Bernardino Ghetti<sup>4</sup>, Pierluigi Gambetti<sup>3</sup>, Byron Caughey<sup>1</sup>\*

# Antisense Oligonucleotides to delay or prevent onset of prion disease in mice

- Grant recipient: Dr. Byron Cau
- Year: 2016



DNA  $\longrightarrow$  mRNA  $\longrightarrow$  Protein



# Antisense Oligonucleotides to delay or prevent onset of prion disease in mice

JCI insight

RESEARCH ARTICLE

## Antisense oligonucleotides extend survival of prion-infected mice

Gregory J. Raymond,<sup>1</sup> Hien Tran Zhao,<sup>2</sup> Brent Race,<sup>1</sup> Lynne D. Raymond,<sup>1</sup> Katie Williams,<sup>1</sup> Eric E. Swayze,<sup>2</sup> Samantha Graffam,<sup>3</sup> Jason Le,<sup>3</sup> Tyler Caron,<sup>3</sup> Jacquelyn Stathopoulos,<sup>3</sup> Rhonda O'Keefe,<sup>3</sup> Lori L. Lubke,<sup>1</sup> Andrew G. Reidenbach,<sup>3</sup> Allison Kraus,<sup>1</sup> Stuart L. Schreiber,<sup>3</sup> Curt Mazur,<sup>2</sup> Deborah E. Cabin,<sup>4</sup> Jeffrey B. Carroll,<sup>5</sup> Eric Vallabh Minikel,<sup>1,3,6,7</sup> Holly Kordasiewicz,<sup>2</sup> Byron Caughey,<sup>1</sup> and Sonia M. Vallabh<sup>1,3,6,7</sup>

**PrProfile: A Study to Assess the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of ION717.**

ClinicalTrials.gov ID ⓘ NCT06153966

Sponsor ⓘ Ionis Pharmaceuticals, Inc.

Information provided by ⓘ Ionis Pharmaceuticals, Inc. (Responsible Party)

Last Update Posted ⓘ 2024-07-03

# Assessing efficacy of zinc-finger repressors of prion protein as a therapeutic for prion disease

- Grant recipient: Dr. Sonia Va
- Year: 202

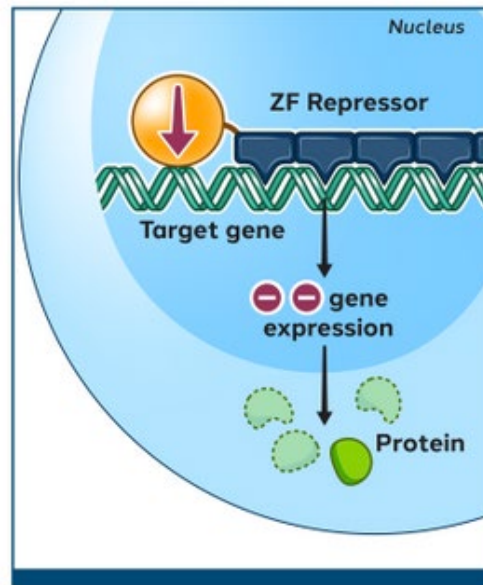


Image source: [www.sangamo.com](http://www.sangamo.com)

# Assessing efficacy of zinc-finger repressors of prion protein as a therapeutic for prion disease

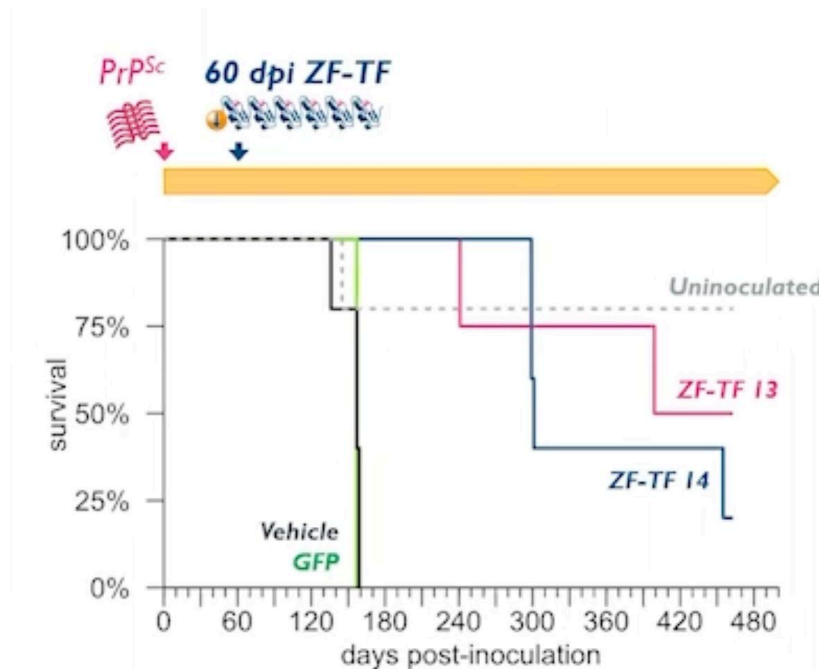


Image source: [www.sangamo.com](http://www.sangamo.com)



## **Take Home Messages**

- Research funded through CJD Foundation Research Grants has led to advances in our ability to diagnose and treat human prion diseases
- Continued funding of fundamental and applied prion research is imperative!
- Research progress is not always linear and can take time
- Prion scientists are extremely grateful to the CJD Foundation as well as friends and family of those affected by CJD for their philanthropic and fundraising efforts that enable these research grants