



**2020**  
**ANNUAL REPORT**

# LETTER FROM EXECUTIVE DIRECTOR

2020. Period. Hard stop. In the annals of history, the year 2020 will be one that stands out. What a challenge it was for everyone, especially members of our Ataxia community. But you showed up. You didn't back down. Your resilience, support for each other, and positivity came shining through. It's what defines this group who all of us at the National Ataxia Foundation have the privilege of helping each and every day. Your support of our work helped make 2020 a truly remarkable year for NAF as well.

Having to cancel two research conferences and our Annual Ataxia Conference at the last moment could have been hard to handle. Instead, NAF's series of webinars that covered the content of AAC were viewed thousands of times. Shifting our popular Walk N' Roll events which take place around the country to an online gathering might have been disastrous for this important fundraising initiative. But you didn't let that happen. Instead, we exceeded our goal and had a lot of fun coming together virtually. Finally, our Ataxia support groups, who so many of you rely on for connection and information, were more important than ever during the year when we couldn't be physically together. You embraced going online, welcoming new members, and becoming Zoom experts all at the same time.

Another good thing happened for NAF in 2020. We updated the organization's vision, mission, and key strategic goals. NAF has been funding basic Ataxia research since our founding in 1957. Those many years of investment have paid off, and now there are a large number of pharmaceutical companies that are pursuing treatments for various forms of Ataxia. It is now time to take a **bold step forward** and start focusing on assisting these companies in any way we can to help them get treatments to market. Asking you all to participate in genetic testing, research studies, and clinical trials will be a crucial part of that help.

We want to thank our dedicated group of generous donors and volunteers for making 2020 so very memorable. NAF is in a strong financial position because of you. You showed up like you always do. Here's to a joyous 2021 and seeing you in person again soon.



Andrew Rosen, *Executive Director*

**Vision:** A world without Ataxia.

**Mission:** To accelerate the development of treatments and a cure while working to improve the lives of those living with Ataxia.

**Strategic Goals:**

- 1) Connect all people affected by Ataxia, both personally and professionally, with NAF.
- 2) Mobilize the Ataxia community to advance and participate in research and clinical trials.
- 3) Enhance local and national support for those living with Ataxia

# 2020 at a Glance



## 2020 AT A GLANCE

27

Research  
Initiatives  
Funded

\$1M+

Total Spent on  
Research

370

Support Group  
Meetings

1,904

New Members  
of NAF

19

Educational  
Webinars  
Hosted

# NAF Membership

As pharmaceutical industry interest in Ataxia continues to grow, our members are integral in moving research forward to get treatments to the market. They are the first to know when there are new opportunities to participate in research or clinical trials for their specific type of Ataxia. NAF had a **52% increase** in members in 2020.



Total members

**5,569**

## ABOUT OUR MEMBERS

3,156

Person with  
Ataxia

283

At-Risk for  
Ataxia

1,624

Family Member  
of Person with  
Ataxia

234

Friend of  
Person with  
Ataxia

272

Ataxia  
Professional

# Community Programs and Services Impact

## SUPPORT GROUPS

NAF coordinates Support Groups as a way for the Ataxia community to connect and learn from others. With the pandemic changing the way that the world gathers in 2020, our Support Group Leaders stepped up to the challenge and found ways to continue to provide this valuable service to our community. Virtual support group meetings allowed us to stay connected from home. Thank you to the volunteers who make it possible.

66

Support Groups

370

Support Group Meetings





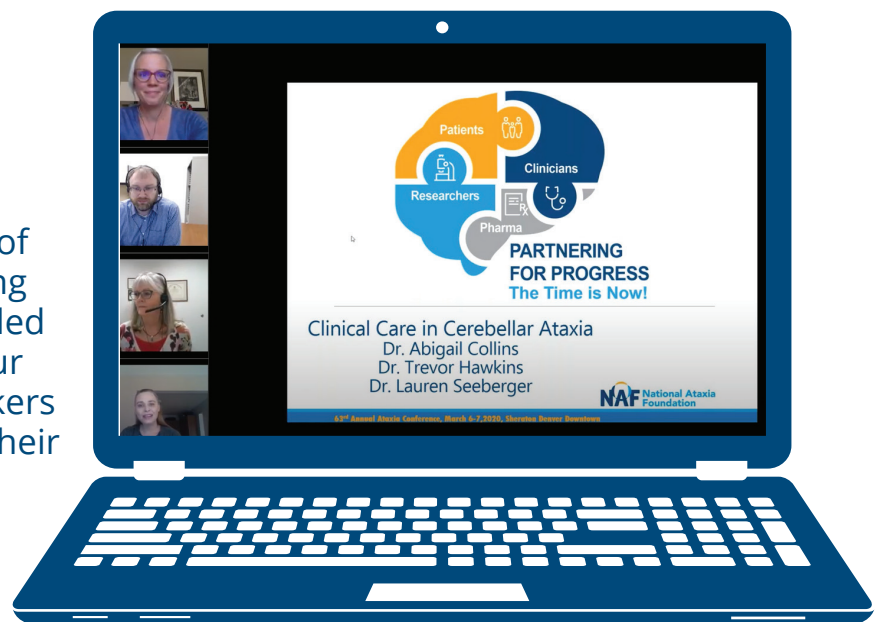
# ADVOCACY

NAF and the Friedreich's Ataxia Research Alliance (FARA) worked together on behalf of Ataxia patients and their families to get support for the National Ataxia Awareness Resolution of 2020 (S.Res. 717). The resolution designated September 25, 2020 as National Ataxia Awareness Day in the United States. The resolution was led by U.S. Senator Elizabeth Warren (D-MA) and U.S. Senator Cindy Hyde-Smith (R-MS) and passed in the US Senate in November of 2020.



# ANNUAL ATAXIA CONFERENCE

For the first time in our 63 years, NAF had to cancel our most beloved event of the year. COVID-19 was in the beginning stages of the pandemic when we decided to prioritize the health and safety of our community. All of our scheduled speakers for the conference agreed to present their topics in a webinar.



# Research Impact

## NAF RESEARCH GRANTS

At NAF, we support researchers through our grant program, scientific meetings, collaborative initiatives, and assistance with research recruitment. We know our participation is crucial to accelerating the developments of treatment and a cure for Ataxia.

**\$1,080,000  
Funded**



### 27 Research Studies Supported

- |  |                                     |
|--|-------------------------------------|
| 9 Seed<br>Money<br>Grants                | 5 Post Doc<br>Fellowships           |
| 1 Pioneer SCA3<br>Translational<br>Award | 2 Special Projects<br>Grant for MSA |
| 7 Young Investigator SCA Awards          |                                     |
| 3 Young Investigator Awards              |                                     |

## RESEARCHER SPOTLIGHT

### Recipient of 2020 Young Investigator SCA award

"Receiving the NAF Young Investigator SCA Award made me very proud. The funding has made it possible to implement a concrete scientific project independently and autonomously...Our research has direct impact on improving trial-readiness for patients. It is a great pleasure and a great enrichment to be able to work in a field that is undergoing such a pivotal period of change... This is an incredible turning point and a strong motivation to continue research and work in this field. Together with Heike Jacobi, I founded the Ataxia Global Young Investigator Initiative. We are happy to share our passion with like-minded people and to pass it on to new young researchers. It has been very motivating to be supported on my scientific journey through funding line of the NAF Young Investigator SCA Award."



**Jennifer Faber, MD**  
German Center for  
Neurodegenerative  
Diseases  
Bonn, Germany

# EXTERNALLY-LED PATIENT FOCUSED DRUG DEVELOPMENT MEETING FOR POLYGLUTAMINE ATAXIAS

NAF partnered with CureDRPLA to host an Externally-Led Patient Focused Drug Development (EL-PFDD) meeting for Polyglutamine Ataxias. An EL-PFDD meeting gives the FDA and other key stakeholders, including medical product developers, health care providers, and federal partners an important opportunity to hear directly from patients, their families, caregivers, and patient advocates about the symptoms that matter most to them, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments.

The Voice of the Patient Report for Polyglutamine Ataxias and DRPLA is a comprehensive document created from the feedback gathered at the meeting. It was submitted to the U.S. Food and Drug Administration. Future applications for therapy

approvals will use the report as a reference to obtain the patient perspective on treatments and to understand what symptoms matter most to patients. This will serve as a guide to Industry as they develop effective and meaningful therapies for the Ataxias.

## MEETING STATS

September 25, 2020

452

Attended Live

1,346

Streamed  
Recording

## ATAXIA COMMUNITY PARTICIPATION

29

Speakers

9

Callers

235

Emailed  
Comments

### THE VOICE OF THE PATIENT: LIVING WITH POLYGLUTAMINE SPINOCEREBELLAR ATAXIAS (SCA) AND DENTATORUBAL - PALLIDOLUYSIAN ATROPHY (DRPLA)



Report of an Externally-Led Patient Focused Drug Development Meeting  
Public Meeting: September 25, 2020 | Report Date: January 2021

**NAF** National Ataxia  
Foundation  
www.ataxia.org

**CURE**  
**DRPLA**





# NAF DRUG DEVELOPMENT COLLABORATIVE

We launched the NAF Drug Development Collaborative (DDC) in 2020. It is a pre-competitive pharmaceutical industry consortium designed to address shared challenges and opportunities in Ataxia therapy development. The Collaborative provides a centralized source of Ataxia expertise, patient registry and natural history data.

## Thank You DDC Members!



## DONOR SPOTLIGHT: THE GORDON AND MARILYN MACKLIN FOUNDATION

The Gordon and Marilyn Macklin Foundation has supported NAF through generous donations to the research program and other initiatives since 2009. Most recently, this support has been directed toward funding of the Clinical Research Consortium for the Study of Cerebellar Ataxia (CRC-SCA). Gordon and his wife, Marilyn, established their foundation to support those who are less fortunate from a health or economic perspective. When the couple passed, Gordon's nephew, Donald (Don) Dawn, assumed the role of the Foundation's President. Marilyn had Ataxia, and thus the focus on supporting those who are also impacted by the condition.

"Thanks to the important work of the CRC-SCA in working collaboratively to collect patient data, we are moving closer to finding treatments and an eventual cure for Ataxia," Mr. Dawn said.

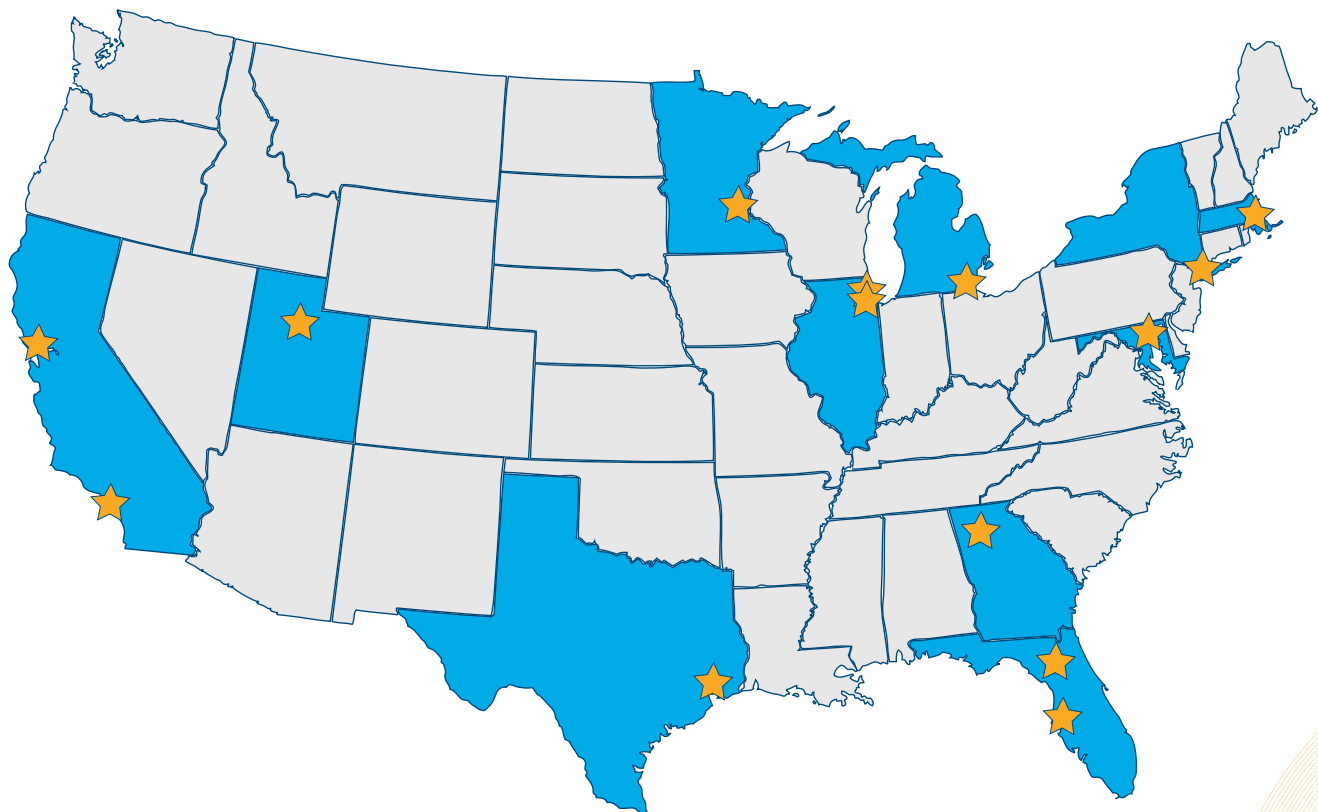
We want to thank the Macklin Foundation for its loyal support of NAF.



# CLINICAL RESEARCH CONSORTIUM FOR THE STUDY OF CEREBELLAR ATAXIA

The CRC-SCA is composed of fourteen Ataxia clinics across the U.S. that specialize in the diagnosis and treatment of Ataxia. These are the leading centers in the country, guided by a distinguished group of Principal Investigators (PI's). The group is conducting a natural history study in Ataxia to better understand the factors that determine disease progression. Their goal is to improve the understanding of the disease process, current treatments, and development of disease-modifying therapies. In 2020, the CRC-SCA consortium began collecting cerebrospinal fluid as a biomarker to better understand disease progression.

- 600+ people enrolled
- Study covers: SCA 1, 2, 3, 6, 7, 8 and 10



**“Continuing natural history data collection will truly make the CRC-SCA clinical trials ready. This is evidenced by numerous pharmaceutical companies expressing interest in both the natural history data, as well as enrolling research participants through CRC-SCA sites.”**

**Vikram Shakkottai, MD, PhD**  
Chair of NAF's Medical and Research Advisory Board

# Revenue and Fundraising



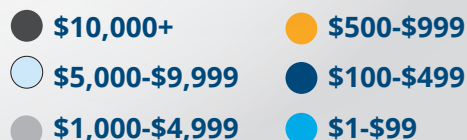
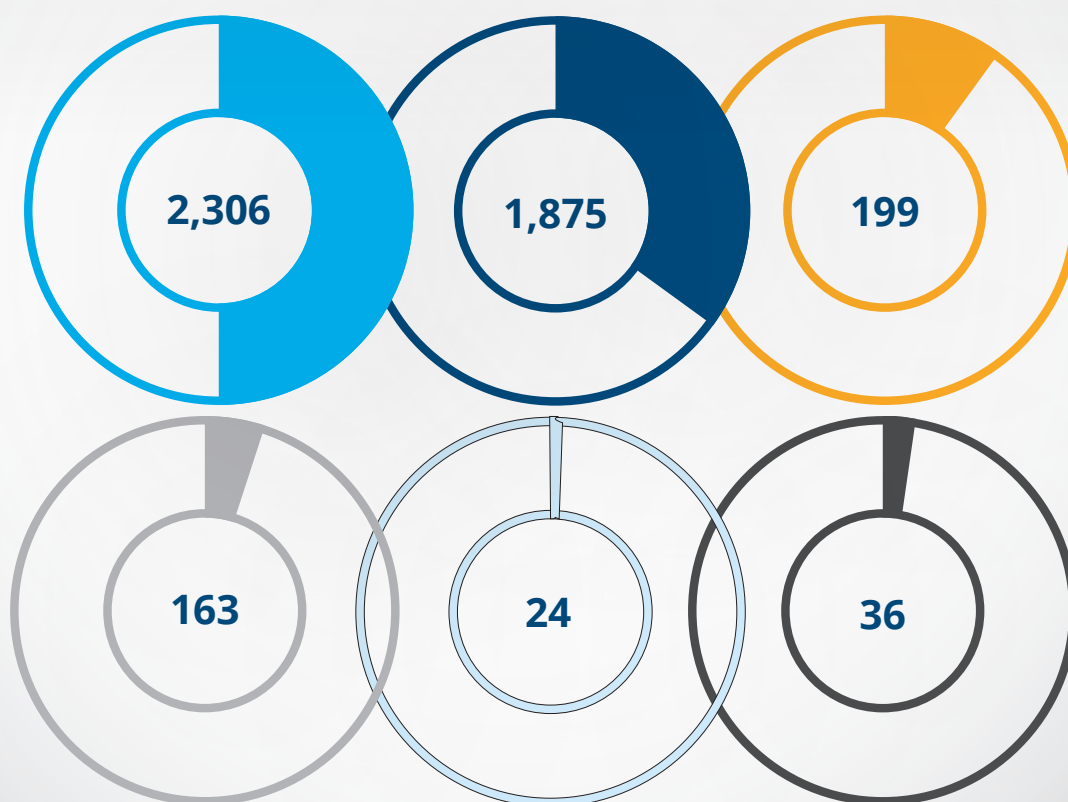
## THANKS TO OUR DONORS!

As NAF takes bold steps forward to accelerate the developments of treatments and a cure, it is our donors that make our work possible. Your generous support allows us to continue to offer community and education programs that empower people with Ataxia and their families to improve their lives.

### DONORS BY GIFT SIZE

Total Donors 4,603

Total Donated \$2,232,007



## CORPORATE PARTNERS

**NAF partners with a variety of corporations. We would like to recognize our corporate partners who provided much needed support.**

### **\$25,000+**

Albert Frei & Sons, Inc.  
Biohaven Pharmaceuticals  
F. Hoffman-LaRoche Ltd.  
Ionis Pharmaceuticals  
Reata Pharmaceuticals Inc.  
Seelos Therapeutics  
Takeda Pharmaceuticals USA, Inc.  
uniQure  
Vico Therapeutics B.V.

Duke University

### **\$5,000 - \$9,999**

APDM, Inc.  
Charter West Bank  
Chondrial Therapeutics, Inc.  
Exicure  
Lacerta Therapeutics  
PTC Therapeutics

Kennebunk Savings Bank  
Live Nation  
Minoryx Therapeutics  
Torres Electrical Supply Co, Inc.

### **\$1,000 - \$2,499**

Acer America Corporation  
Cascade Drilling  
ChronoTrack Systems Corp  
First Eagle Investment  
Los Alamos National Security LLC  
Preferred Title

### **\$10,000 - \$24,999**

Biogen  
Cadent Therapeutics

### **\$2,500 - \$4,999**

Eagle Ridge  
Gullford Robinson Charitable  
Account

## FOUNDATIONS

**NAF received grants from various foundations in 2020. We thank them for their support and commitment to our mission.**

### **\$25,000+**

Cossack Foundation  
Gordon & Marilyn Macklin  
Foundation  
The Clementz Foundation

### **\$5,000 - \$9,999**

Brett and Melody McKay  
Leader Family Foundation  
The Bob Allison Ataxia Research  
Center

### **\$1,000 - \$2,499**

Bierley Charity  
Elsevier Foundation  
Enterprise Holdings Foundation  
EveryLife Foundation  
Fidelity Charitable  
Greater KC Community  
Foundation  
Marcie L Anthone Charitable Fund  
The Evelyn S & Jim Horne Hankins  
Foundation

### **\$10,000 - \$24,999**

CureDRPLA  
The Tom, Mary, and Becky Babcox  
Family Foundation

### **\$2,500 - \$4,999**

Ataxia UK  
Nebraska Ataxia, Inc.

## COMMUNITY GIVING PLATFORMS

**NAF does not receive individual donor information from third-party giving platforms. We do want to recognize the individuals who supported us through a community giving platform. Thank you for your generous contributions in 2020!**

### **\$100,000+**

Benevity Community Fund  
Facebook

### **\$2,500 - \$4,999**

Amazon Smile Shopping Program  
Charities Aid Foundation of  
America

### **\$1,000 - \$2,499**

Cybergrants 3M Foundation  
Paypal Giving Fund  
Shell & Motiva Employee Giving  
United Way of Chicago Area

### **\$5,000 - \$9,999**

CFC TASC  
Chesapeake Chapter of NAF  
Community Health Charities  
Mississippi Chapter of NAF  
United Way of the Capital Region



# INDIVIDUALS

We are humbled by our generous donors who supported our work in 2020. Despite the uncertainty in the world due to the pandemic, these individuals stepped forward to make sure that NAF can continue to lead efforts to accelerate development of treatments for Ataxia. The caring individuals who contributed \$1,000 or more are listed below.

## \$50,000+

Anonymous  
Anonymous  
Anonymous  
Joseph Frei  
Marcia Neugebauer  
Terrence Fort Estate

Katherine Sweeney  
Mike and Toni Rosen  
Noorin Bhanji  
Roger and  
Elaine Wheeler  
Ronald Mason  
Sergio Damasio  
Sylvia Shelstad  
Tim and Karen Miller

Georgia Trujillo  
Gerald Matykowski  
Gerard D. Hont  
Greg Rooks  
Gregory Messigian  
Harlan Lindberg  
Harriet Rosen  
Isabella Carvara Jr.  
Jane and Larry Jaffe  
Jason Dillon  
Jason Pruet

Matt Curran  
Melissa Clausen  
Michelle Serrano  
Mike Estabrook  
Nancy Haugen  
Nancy Schultz  
Neal & Kath Verga  
Patricia Thomas  
Paul & Susan Graseck  
Paul Elmore  
Perry Giles  
Priscilla Wallack  
Ray and Jane Dunkelberg  
Rebecca Lipner  
Renee DuShane  
Rhonda Holt  
Richard and Carole Carr  
Richard Jones  
Robert Baker  
Robert Hartmann  
Ronald Phelps  
Ross Way  
Roy Francis O'Connor  
Samuel Kirton  
Sandra Deane  
Sandra Johnson  
Sandy Miller  
Socorro Cavazos  
Susan Huffman  
Tamara and  
Steve Peterson  
Teann Nash  
Terrance Carr  
Terry and Cynthia Kinney  
Terry Dougan  
Thomas Jones  
Thomas Malia  
Valerie Arias  
Vicki Merrill  
Virginia Lucas  
William Pierro

## \$25,000 - \$49,999

Kathleen and  
John Schnobrich

## \$1,000 - \$2,499

Amy Lau  
Anna Matykowski  
Anthony and  
Robyn Zeller  
Antionette Alvarez  
Barbara and Frank Tinari  
Bernard Frei  
Bill and Janet Nye  
Bill Laine  
Bob Sturdevant  
Brad Delano  
Bruce Ruehl  
Carolyn Pierce  
Cathleen and  
Rick Bethay  
Cecil Roberts  
Charles Aimone  
Charlotte Depew  
Christian Maier  
Daniel & Laurie Flynn  
Daniel and Sheila Irwin  
Dave and Karen Henry  
Debbie Ringer  
Dick Manley  
Don & Cynthia Lara  
Donald and  
Sherrie Hansen  
Dorothy Bowdon  
Earl McLaughlin Jr.  
Edgardo Rodriguez  
Edith Payne  
Edward and  
Susan Ciolkosz  
Edward Levin and Nancy  
Miller-Levin  
Edward Tinkham  
Elizabeth White Shainline  
Eric and Amy Swierczek

Jay and Meg Larson  
Jeannette and  
Matthew Viveiros  
Jeff Stark  
Jennifer Gutwein  
Jenny Parker  
John Brittain  
John Teget  
Judith Chronister  
K. C. and Nancy Turnbull  
Karter Edwards  
Katharine Adams  
Kenneth Kueny  
Kenneth Neff  
Kerry Enright  
Kevin Forrest  
Krisie Nave  
Laura Ospanik  
Lauren Anthone  
Laurie Duncan  
Lawrence Adair  
Leah and Mark Minkin  
Linda Snider Sidwell  
Lucille Lundquist  
Maggie & Wally Ducayet  
Marcia Murto  
Margaret Kennedy  
Mariam Bahmane  
Marilyn Teske  
Mark and  
Laneta Graham  
Mark and  
Rebecca Massman  
Mark Robinson  
Mary Ann Tucci  
Mary Bird  
Maryann Sweeney and  
John Dyer

## \$10,000 - \$24,999

Anonymous  
Anonymous  
Anonymous  
Gregory Klassen  
Jean Booher  
Jennifer Warchol  
Judy Meelia  
Lisa Frei  
Ruth Coffey  
Thomas Frei

## \$5,000 - \$9,999

Bob Schlickbernd  
Cherie Nixon  
Darrell Knutie  
Douglas and  
Kimberly Brunnert  
James Keene  
Laura Ranum PhD  
Mark Pejokovich  
Russel Derickson  
Tamra Humes  
Thomas Keene  
William Nye  
William Sweeney

## \$2,500 - \$4,999

Alex and Marlys Meyer  
Andrea Matarazzo  
Andrew Pickering  
Anonymous  
Barton Suchomel  
Brian Covington  
Janet, Mike, and  
Brynn Dyer  
Karen Brown



# VIRTUAL WALK N' ROLL TO CURE ATAXIA

For the first time since Walk N' Roll to Cure Ataxia began in 2007, a single national event was held virtually! Our event organizers were not able to put together in-person Walks in their communities, so they joined us online for live-streamed entertainment and fun instead. Thank you to all who attended, fundraised, and/or donated to the campaign!



National Ataxia Foundation

October 3, 2020

**414** ▶ Attended Live

**\$298,065** Raised

**2,032** ▶ Donations



# PASSION FUNDRAISING

Many thanks to the individuals or groups who turned their passions into a community fundraising event for a good cause! These events raise money in support of NAF's mission to accelerate the development of treatments while improving the lives of those living with Ataxia. Thank you for the generous donations and time spent organizing the events.

**2** ▶ Passion Fundraisers

**\$28,882** ▶ Raised

**\$132,741** Joint Mission Bataan

# WORKING TOWARDS A CURE

## SUPPORT & REVENUE

Contributions	\$2,232,007
Research Collaborative	\$330,000
Conference Income	\$240,732
Other Income	\$187,786
<b>Total</b>	<b>\$2,992,089</b>

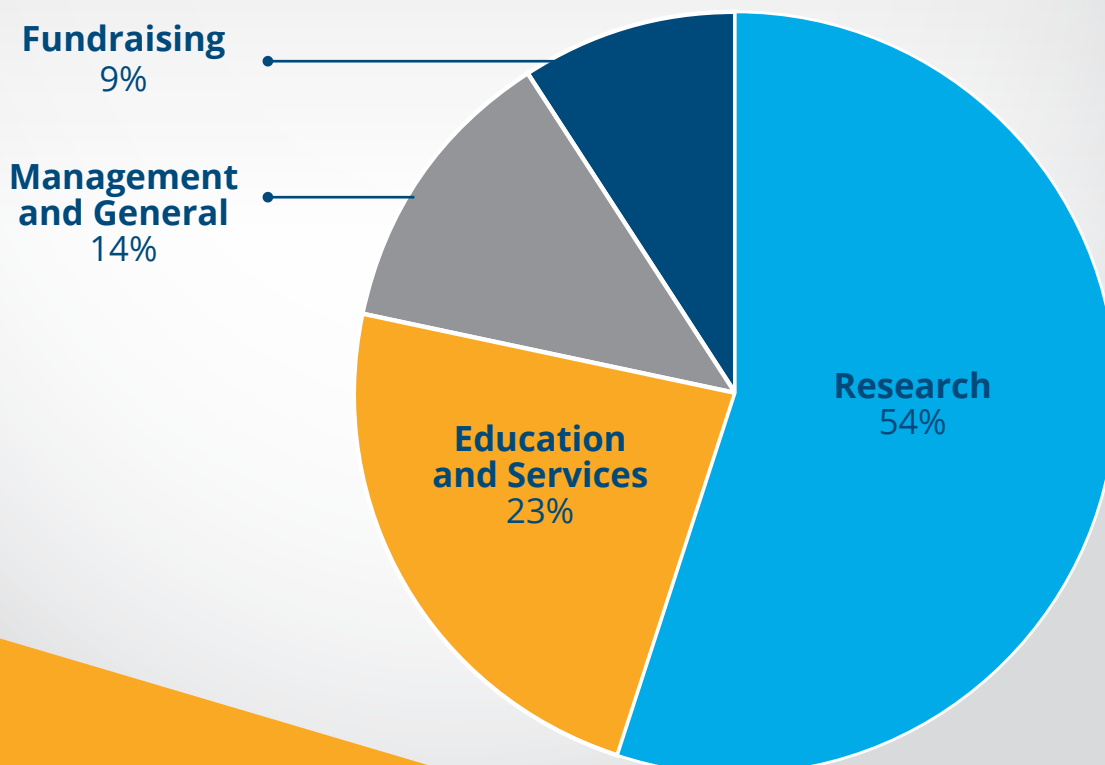
## EXPENSES

Research	\$1,518,362
Education and Service	\$646,191
Management and General	\$391,273
Fundraising	\$248,130
<b>Total</b>	<b>\$2,758,956</b>

## NET ASSETS

Change in Net Assets	\$264,465
Net Assets, beginning	\$3,597,738
Net Assets, ending	\$3,862,203
Total Assets	\$4,138,714
Total Liabilities	\$276,511
Total Net Assets	\$4,138,714

## HOW WE SPEND OUR MONEY





# Organization Leadership

## Board of Directors

### President

Sam Kirton  
Fairfax, VA

### Vice President

Linda Snider-Sidwell, MD  
Omaha, NE

### Treasurer

Camille Daglio  
Hattiesburg, MS

### Secretary

John Mauro  
Auburn, MA

### Executive Committee Member

David Brunnert  
Houston, TX

### Directors

Michael Cammer  
Downington, PA

Joseph DeCrescenzo  
Bear, DE

Cindy DeMint  
Yorba Linda, CA

Mike Leader  
Hummelstown, PA

Greg Rooks  
Atlanta, GA

Wilson Romero  
New Iberia, LA

Peter Schnobrich  
Minneapolis, MN

Bill Sweeney  
Minneapolis, MN

### Ex Officio Board Member

Harry T. Orr, PhD  
Minneapolis, MN

Susan Perlman, MD  
Los Angeles, CA

Laura Ranum, PhD  
Gainesville, FL

Larry Schut, MD  
Maple Lake, MN



*Thank you!*

**Thanks from the NAF Staff!** To our donors, community members, partners, and Ataxia professionals, we thank you for making 2020 a successful year with much progress made towards NAF's mission. Your support and participation are key to accelerating the development of treatments for Ataxia.

## Medical and Research Advisory Board

### Chair of MRAB

Vikram Shakkottai, MD, PhD  
University of Michigan

### Research Director

Harry T. Orr, PhD  
University of Minnesota

### Research Director Elect

Laura Ranum, PhD  
University of Florida, College of Medicine

### Medical Director

Susan Perlman, MD  
UCLA Neurological Services

### Medical Director Elect

Liana Rosenthal, MD, PhD  
Johns Hopkins University

### MRAB Directors

Tetsuo Ashizawa, MD, FAAN  
Methodist Hospital Research Institute

Khalaf Bushara, MD  
University of Minnesota

Beverly Davidson, PhD  
Children's Hospital of Philadelphia

Brent L. Fogel, MD, PhD  
University of California, Los Angeles

Christopher M. Gomez, MD, PhD  
University of Chicago

Pravin Khemani, MD  
Swedish Neuroscience Institute

Sheng Han Kuo, MD  
Columbia University

Albert La Spada, MD, PhD, FACMG  
University of California Irvine School of Medicine

Puneet Opal, MD, PhD  
Northwestern University Feinberg School of Medicine

Gülin Öz, PhD  
University of Minnesota Center for Magnetic Resonance Research

Henry Paulson, MD, PhD  
University of Michigan Medical Center

Stefan Pulst, MD  
University of Utah Health Sciences Center

Jeremy D. Schmahmann, MD  
Harvard Medical School

Larry Schut, MD  
University of Minnesota

S.H. Subramony, MD  
University of Florida

George "Chip" Wilmot, MD, PhD  
Emory University

Robert B. Wilson, MD, PhD  
University of Pennsylvania



600 Highway 169 South, Suite 1725  
Minneapolis, MN 55426  
(763) 553-0020 • [naf@ataxia.org](mailto:naf@ataxia.org) • [www.ataxia.org](http://www.ataxia.org)  
Tax ID: 41-0832903