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National Ataxia Foundation

GENERATIONS

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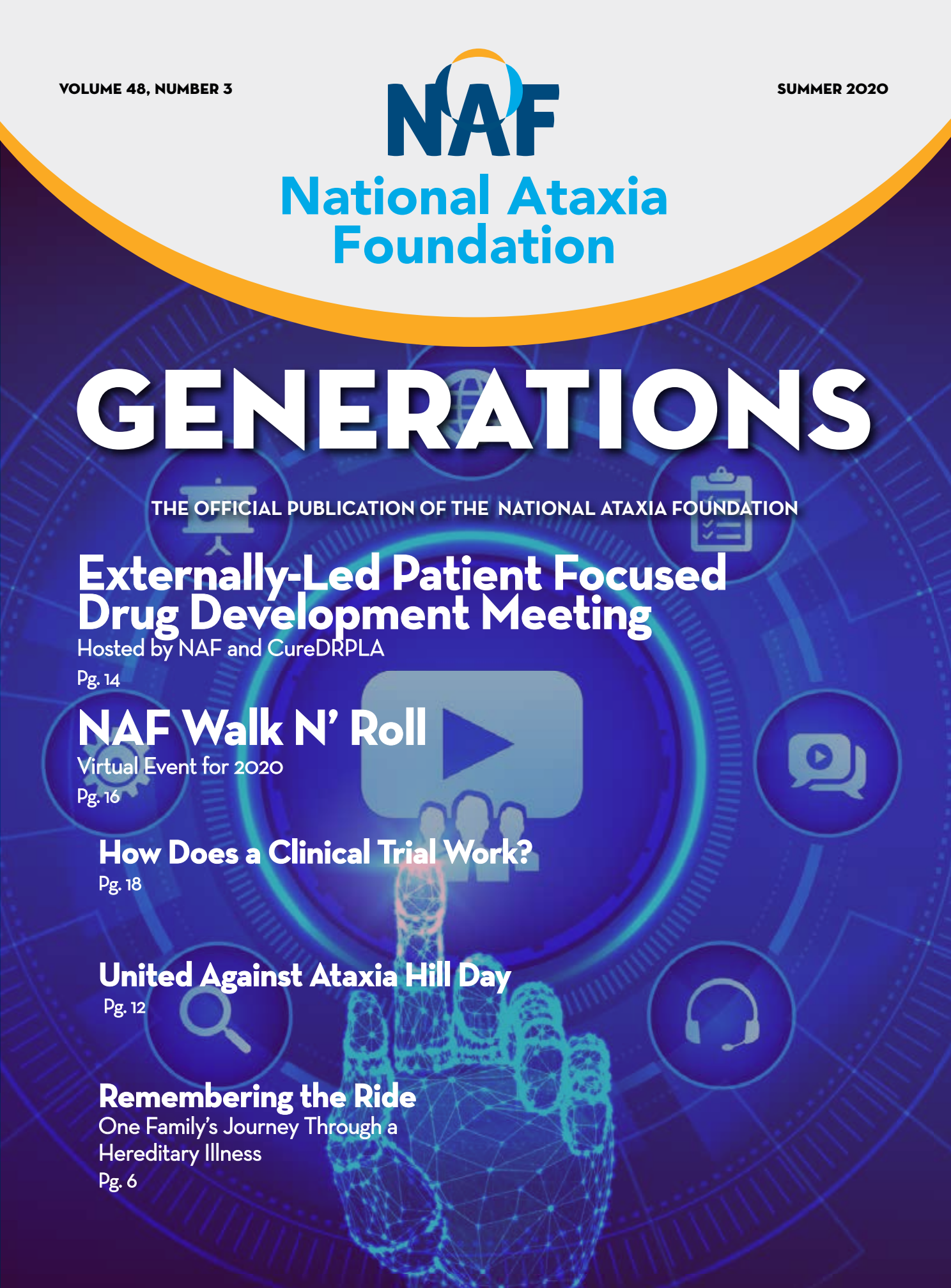

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Deadline to submit materials for the Fall issue of *Generations* is September 10, 2020.

Please direct correspondence to:

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CFC #10752

Generations Schedule:

Issue	Spring	Summer	Fall	Winter
Mail Date	April	July	October	January

NAF Update:
Letter from the Executive Director

Andrew Rosen,
Executive Director

Hello NAF Community:

As the calendar turns to September (and my kids "sort of" go back to school), I will admit that I have grown tired of my home office. I miss the daily camaraderie of the NAF staff, and the chance to travel to visit support groups, meet donors, and participate in fundraising events. But I'm always looking for the silver lining these days. You all provide the motivation to make lemonade out of lemons, and it's amazing what we can accomplish by thinking creatively and virtually connecting with each other.

I'd like to highlight one of our recent successes, and what we have coming up for a busy late summer and fall:

1. Your generosity never ceases to amaze! I'll admit a bit of trepidation when we launched our Summer Match Challenge, but you handily beat our goal of $100,000, which was matched by the Michael and Patricia Clementz-Peterson Family Fund and an anonymous donor. Thank you!

2. The pandemic cannot stop us from hosting our second "United Against Ataxia Hill Day," our annual advocacy effort in partnership with FARA (Friedreich's Ataxia Research Alliance). We will be meeting online with many Senate staffers on September 10th to continue to push for causes that positively impact our community.

3. Our Externally-Led Patient Focused Drug Development Meeting (that's a mouthful!) will be held online on September 25th. This is a milestone event for NAF as our community will have the chance to share their stories directly with FDA staff, detailing how Ataxia impacts their daily lives. You are all invited to join us for this important event in the drug development timeline

4. Lastly, our first ever Virtual Walk N' Roll event is scheduled for October 3rd. We're working on a great program for you, and we hope you'll join us for our largest fundraising event of the year!

More details will follow on all of these activities. In the meantime, thank you for your continued support of our work at NAF. I wish you all a low-humidity, mosquito-free, sun-filled rest of summer.

ISSUE HIGHLIGHTS

NAF Funded Research

A No-Cost Extension was issued for the following 2018 NAF funded grant award, resulting in it being printed in this publication rather than the Fall 2019 edition.

RESEARCH SEED MONEY AWARD

Diane M. Papazian, PhD | UCLA | Los Angeles, CA

Phenotypes of Early- and Late-Onset SCA13 Mice

Spinocerebellar ataxia 13 (SCA13) is caused by missense mutations in the KCNC3 gene, which encodes the Kv3.3 voltage-gated potassium channel. Potassium channels are proteins that control the flow of potassium ions across cell membranes. These channels contribute to the electrical signaling that is characteristic of brain and muscle. The Kv3.3 channel is highly expressed in cerebellar neurons, including Purkinje cells, where it is a key regulator of action potential firing. This suggests that mutations that cause SCA13 alter the function of cerebellar neurons, which may contribute to the clinical characteristics of the disease. Unlike most SCAs, SCA13 exists in two forms depending on the mutation. An infant-onset form is characterized by cerebellar maldevelopment and atrophy early in life, motor delay, and persistent motor deficits. An adult-onset form typically emerges in the third or fourth decade of life with progressive ataxia and progressive cerebellar degeneration. Distinct mutations in the KCNC3 gene cause the two forms of the disease. We generated three lines of genetically altered mice in which the normal Kcnc3 gene has been replaced by an allele that causes SCA13 in human patients. Two of the lines carry infant-onset mutations, while the third carries an adult-onset mutation. With support from the National Ataxia Foundation, we have begun to characterize the phenotypes of these mouse models. Our results to date indicate that the infant-onset mutations cause severe locomotor impairment soon after birth. This is accompanied by the loss of cerebellar Purkinje cells and is correlated with abnormalities of electrical signaling in the cerebellar circuit. In contrast, mice with the adult-onset mutation appear normal early in life. Experiments are ongoing to determine whether phenotypes arise as the animals age. Our results indicate that the SCA13 model mice provide an opportunity to identify mechanisms that underlie the symptoms of the disease and to explore treatments that could improve locomotor function and reduce cerebellar neurodegeneration in these animals. The ultimate goal is to identify therapies that may be helpful for SCA13 patients.

Visit www.ataxia.org/naf-research for more information on:

- **Past Funded Research Projects**
- **Research Participation Opportunities**
- **Tissue Donation Program**
- **Patient Registries**

What is the Deal with Ataxia Research Right Now?

Tasha Kaiser, Clinical Research Coordinator, University of Michigan

Hello, with a big friendly wave of the Michigan mitten! My name is Tasha Kaiser, and I am the Clinical Research Coordinator for the University of Michigan Ataxia studies. I know that so many of you in the Ataxia community have unrelenting questions about the current status of research and how the COVID-19 restrictions impact it, so I wanted to share some enlightenment on the subject and hopefully take some of that edge off for you! But first, I want to say a few things:

- **Your safety is our number one concern!** While the research coordinators miss seeing you in person, we want to make sure that we are doing everything we can to keep you safe and healthy! Unfortunately, that means making sure you can do most things from the comfort of your home (like virtual visits) and away from areas that would increase your exposure!

- **We are hear for you if you need anything!** It's during difficult times like these that you may find yourself in a place that is not good for your well-being. This is normal but important to recognize. Please reach out for assistance in alleviating whatever ails you and know we are here to help. We have an abundance of resources to fit almost any need and are just a phone call or email away!

Ataxia Research During COVID

Now, the good stuff! Here is what is going on with research:

- **Research IS still open and ongoing!** While most interventional trials are now doing remote visits and most observational studies have paused, the research teams are still working hard behind the scenes. We are taking the opportunity to make sure all of our studies are in pristine shape and making preparations to get all participants back in as soon as we can!

- **We would love to at least do a virtual visit for all studies, but we have decided that it's better to pause the observational studies until we can see you in person.** The research on Ataxia is so precious, and to ensure that we collect the best data we know that completing certain study assessments in person is the most accurate way to do so. So please do not think observational studies are any less important just because they are not having virtual visits!

- **Your study team might have reached out to you, but if they have not don't hesitate to contact them with your questions!** This has been a big learning curve for everyone, and most of us have had to adjust to working remotely. We do not mean for it to seem as if we have forgotten about anyone, but we may get wrapped up in making sure we manage these changes properly! So please contact your coordinator if you have any questions or concerns!

At this time everything is dynamic and we are just going with the flow as best we can. We are doing everything in our power to plug along and prepare for what lies ahead. I wish I could say we knew, but we must be ready to adjust if changes occur, which they very well may. I like to think that these changes will be for the betterment and that one positive outcome of this pandemic is that it will make research more resilient! We need to stay strong, stay positive, and stay together!

Lastly, I just want to remind everyone that each state is different regarding restrictions, and that they vary from institution to institution. Some research sites may be able to see participants before others and it is vital to be patient with the process of reopening! Again, safety is our number one concern and we strongly encourage everyone to follow the guidelines of the CDC and state officials! But please know that you can reach out to us at any time- we would love to hear from you as we too are missing the social interaction!

Remembering the Ride
A Memoir — One Family's Journey Through a Hereditary Illness

Shirley Swier Jones

Shirley Swier Jones was caregiver for her husband, Vernon for many years as he struggled with Spinocerebellar Ataxia. She and Vernon had five children, all who were at risk for this disease. Shirley filled the primary role of parenting their children throughout the years when Vernon could not be fully involved due to his illness.

This story is a personal one. In it, Shirley takes the reader through fifty plus years of this family's journey through a devastating disease. It is a story of deep sorrow mingled with the joy that comes from family, love, commitment, and faith. It is Shirley's prayer that telling this story will give other families living with disease, either in themselves or a loved one, and all that means in their lives—strength, courage, and faith to face the challenges each day presents.

Available through Amazon, Kindle, Barnes and Nobel and in Bookstores everywhere through Ingram Spark.

Ataxia Tips

for Ataxians... from Ataxians

For many with Ataxia, everyday tasks can become increasingly difficult. One person's shared tip might just be the help someone else needs. See what advice Ataxians are giving to make everyday tasks easier...

Ataxia Tips must be submitted by 9/10/20 to be eligible for inclusion in the next issue of *Generations*. Submit them via email to naf@ataxia.org.

There are lots of exercises for those who can stand or walk, but what about those who can't? Here are some tips to try. The number of reps depends on your ability and challenges. The following exercises can be done with a stationary or recumbent bike.

1. While holding on to the handle bar with one hand, lift the opposite knee towards your chest and vice versa.
2. Hold on to the bar and stand up, lift each knee up to the bar.
3. Sit down, hold on to the bar, stand up. While standing, raise up your heels, then sit back down.
4. Peddle your bike.
5. Grab the bar, lift yourself to a standing position, stay there and enjoy the fact you are standing, slowly lift your hands off the bar. You're standing on your own, if only for a few seconds.

Submitted by DS Chip Carroll, Jr.

NAF recommends that you check with your doctor or physical therapist before trying any new exercises.

KNOW THE FACTS ABOUT SPINOCEREBELLAR ATAXIA AND A NEW CLINICAL STUDY

About Spinocerebellar Ataxia (SCA)

SCA is an inherited form of ataxia, a rare and progressive neurological disease that develops due to damage to the cerebellum, the part of the brain responsible for coordinating movement.

Ataxia affects nearly 150,000 people living in the U.S. of which an estimated 3,200–18,000 have SCA

Symptoms of SCA may include lack of coordination, trouble with balance, difficulty swallowing, slurred speech and/or deterioration of fine motor skills

The most common types of SCA are SCA1, SCA2, SCA3, SCA6 and SCA7 which are caused by specific genetic defects

People are typically diagnosed in their mid-30s but SCA can affect all ages, genders and race

There is no cure or FDA-approved treatment for SCA

Current treatment approaches focus on symptom management to improve quality of life

SCA Study Overview

The Phase 3 randomized, controlled study is designed to evaluate troriluzole, an investigational drug that modulates the brain chemical glutamate. Brain cells communicate with each another by using chemicals, such as glutamate.

Participants are enrolled in the study for 48 weeks*

** Participants who participate in the study will be eligible to continue for an additional 48 week phase where all participants receive troriluzole.*

Participants are randomized one-to-one on troriluzole or placebo and take two pills once daily

18+ More than 18 U.S. medical centers are participating in the study

Mainly focus on disease-types SCA1 and SCA2

Study will measure if troriluzole can slow down and improve ataxia symptoms in people with SCA

Primary endpoint = measureable change in ataxia symptoms, including walking, standing, sitting and speech

Key Eligibility Criteria

- Known or suspected diagnosis of SCA1 or SCA2
- Confirmed clinical evidence of SCA diagnosis or willingness to have testing completed
- Ability to walk eight meters without human assistance (canes or other devices are allowed)
- Be physically able to complete the trial (adequate hearing, vision and language skills)

For more information about this study, visit
www.scatrial.org

The study is sponsored by Biohaven Pharmaceuticals.

MICHIGAN MEDICINE
UNIVERSITY OF MICHIGAN

SPINOCEREBELLAR ATAXIA TYPE 3 (SCA3)

UNIVERSITY OF MICHIGAN RESEARCH OPPORTUNITY

Do you or a family member suffer from SCA3?

The Neurology Department of the Medical School is currently conducting a research study for the purpose of identifying new biomarkers useful for SCA3, and your help is needed!

We are looking for:

- Healthy individuals and individuals with known SCA3 over 18 yrs. of age
- Approximately 1 hour of your time
- Involves a single lumbar puncture and blood draw
- Paid incentive for participation

Some exclusions apply - contact us at
(734) 232-6247

REMEMBERING NAF IN YOUR WILL

Throughout the years, individuals have named the National Ataxia Foundation as a beneficiary in their wills. Their thoughtfulness and foresight has enabled NAF to provide more research studies, more services to patients and families and more education and Ataxia awareness to the public. We are grateful for the impact that has been made by these compassionate acts. If this is something you would like to consider, please contact Joel Sutherland at *joel@ataxia.org* or call (763) 231-2748.

TO SEE THE LATEST ATAXIA NEWS:

- Research
- Community Events
- Membership
- Support Groups
- Fundraising
- NAF Updates

Visit: ataxia.org/blog

2021 AAC Goes Virtual

We know how valuable it is for those living with Ataxia and their caregivers to come together in person at our annual conference to gain strength from shared experiences. The current COVID-19 pandemic necessitates a new approach to many aspects of life—particularly those that involve gathering together in large groups. After much consideration and input from our community and NAF's Medical and Research Advisory Board, **we have decided to move forward with plans to hold the 2021 AAC in a virtual format.** Our priority is to ensure the welfare of all attendees. In addition, many months of planning go into holding AAC, and NAF has to make financial commitments to the hotel and other vendors well ahead of the event itself. We believe this is the right decision to make for our community. Please know that we are committed to holding in-person events again as soon as it is entirely safe to do so.

Now for the good news!

By creating a virtual environment for AAC, the conference will be accessible to all, regardless of ability or willingness to travel. We will be leveraging the expert knowledge of our community in developing the infrastructure needed to make the 2021 AAC an outstanding experience. Opportunities to share your feedback will be offered over the next few months. We will maintain our conference objectives: networking, education, information, inspiration, and fun! The conference will continue to address the many impacts that the community faces, especially during these unprecedented times.

Thank you for your patience and support as we continue to work out the details on this change. Stay tuned!

Lori Shogren, Community Program and Services Director, NAF

What is the CoRDS Registry?

Submitted by Alyssa Mendel

The National Ataxia Foundation has had a long-standing partnership with the Coordination of Rare Diseases at Sanford (CoRDS) Registry for over 7 years. The CoRDS Registry coordinates the advancement of research into 7,000 rare diseases via data sharing and study recruitment. CoRDS works with advocacy groups such as NAF, individuals who have a diagnosis of Ataxia, and researchers who are studying various forms of Ataxia.

Participating in CoRDS gives patients the chance to contribute to research. Information from the registry may lead to better physician decisions and diagnoses, along with better industry models for drug development.

You may be wondering, "What exactly is a patient registry?" A patient registry collects data that includes basic demographic information, health information, quality of life information, and specifics about a particular condition, such as different forms of Ataxia. A registry can serve as a way to contact members of a group later on, or it can serve to build up a story of the progression of a disease/condition, called its Natural History.

Participants can enroll in CoRDS by completing a questionnaire that was created in partnership with the NAF. Researchers and clinicians can then apply for access to receive the de-identified data through the Sanford CoRDS and NAF's Medical and Research Advisory Board. The de-identified information helps researchers when they are working on treatments, therapies, and trying to gain a better understanding of what is going on with a condition. Researchers will also request

Have you joined the Ataxia Patient Registry yet?

CoRDS is a centralized international patient registry for all rare diseases; it is based at Sanford Research. The goal of the CoRDS registry is to connect as many patients and researchers as possible to help advance treatments and cures for rare diseases. The CoRDS registry is free for patients to enroll and is available for researchers to access to recruit research participants.

Since 2013, NAF has partnered with CoRDS to enroll participants who have a diagnosis of Ataxia or are at-risk for Ataxia. Since that time, several researchers have accessed the Ataxia registry to help recruit research participants for their important studies.

If you have not enrolled yet, you can register today to help researchers find more answers to Ataxia and to participate in research studies and clinical trials.

CoRDS

SANFORD RESEARCH

Enroll at www.sanfordresearch.org/SpecialPrograms/cords

Questions? Contact CoRDS at cords@sanfordhealth.org or (877)658-9192

that CoRDS contact participants on their behalf about upcoming clinical trials or studies. After a participant has given consent to share data, patient advocacy groups can use data for non-research purposes, such as for education and grant applications.

Another common question participants have is, "Who owns the data at CoRDS?" Participants are the primary owners of the data because they control how the data is ultimately used. CoRDS simply stores the information as required by the Sanford Research Internal Review Board (IRB). CoRDS will never sell, rent, or lease personal information.

So, how can you enroll today? By going to www.sanfordresearch.org/SpecialPrograms/cords, you will be brought to the Activation Form. This simple form helps to set up your account with the CoRDS Registry. After you have completed the Activation Form, you will be able to complete questions specific to your condition either online in your account or by mail. If you choose mail based participation, CoRDS staff will be able to enter your data into the secured registry on your behalf.

In order to study the natural progression of a rare condition it is critical that participants update their information as frequently as their condition changes. Once a year, CoRDS will remind you to update any information or changes in symptoms. You, of course, are welcome to log into your account or request a new questionnaire at any time to update information.

If you have any questions or need help enrolling, give CoRDS a call at 1-877-658-9192 or email us at cords@sanfordhealth.org

We would be more than happy to assist in any way we can!

The National Ataxia Foundation (NAF) and FARA will be holding their second Hill day on Thursday, September 10, 2020 to raise awareness about Ataxia and support legislation that directly impacts our communities. In addition to the scheduled meetings NAF and FARA will have with US Senators, we are asking members of both communities to flood the hill with correspondences (calls, emails, social media posts) that day! Here is how you can take part!

Become an Ataxia Advocate

Visit our Advocacy webpage www.ataxia.org/advocacy for advocacy tools, events, and resources.

On the advocacy webpage you can:

- Download the Advocacy Toolkit
- Learn how to contact your local representatives to request a Proclamation for International Ataxia Awareness Day (IAAD).
- Check out advocacy events happening around the country.
- Get the latest news on legislative issues impacting the Ataxia community.

We want to hear from you if you have a relationship with a US Congressman or Senator to further support the legislative issues impacting the Ataxia community.

Save the date to join an advocacy webinar on Wednesday, September 2 at 7 pm EDT.

Members from NAF and FARA will review our priorities and review how you can make your voices heard.

A quick highlight of the issues we are currently following are:

- **National Ataxia Awareness Day Resolution:** International Ataxia Awareness Day is celebrated on September 25. NAF and FARA are asking for a resolution to also recognize that date as National Ataxia Awareness Day. Designating September 25 as National Ataxia Awareness Day will elevate the awareness needed to improve the lives of the persons and families affected by Ataxia and can help accelerate development and access to effective treatments.
  - **A Republican Co-Sponsor is Needed for our Resolution!** If you have a Republican Senator in your state please Contact them about Co-Sponsoring this Resolution. Please contact Lori Shogren at lori@ataxia.org if you have any questions or potential Republican candidates.
- **Increased funding for the NIH and FDA:** Elected officials will be working on appropriations for our government partners at the NIH and FDA in September. These agencies are vital to our drug development program and need the resources to appropriately manage the demands of COVID in addition to all the other important research programs they review.
- **Community Home Health and Telehealth Policies:** In the early weeks and months of the COVID-19 public health emergency, Congress and the Administration took important steps to ensure patients have access to essential care while the nation grappled with controlling the spread of the virus. Specific time-limited regulatory flexibilities have removed significant barriers to care and improved access for the 30 million Americans living with a rare disease or condition including Ataxia. However, these flexibilities are at risk of going away when the public health emergency ends. We urge policymakers to recognize how these flexibilities have benefited members of the rare disease community and consider which policies should be kept in place after the public health emergency ends.
- **Creating Hope Reauthorization Act:** This bill will permanently authorize the Pediatric Priority Review Voucher (PRV) program which will further the opportunity to spur innovation in rare and neglected diseases that disproportionately impact children.
- **Rare Disease Congressional Caucus:** The Rare Disease Congressional Caucus helps bring public and Congressional awareness to the unique needs of the rare disease community (including patients, physicians, scientists, and industry), and creates opportunities to address roadblocks to the development of and access to crucial treatments. The Caucus gives a permanent voice to the rare disease community on Capitol Hill. Find Out if your Representatives are members of the Rare Disease Caucus. Thank your Representatives that are members and invite those that are not to join.

For more information contact Lori Shogren, Community Program and Services Director at lori@ataxia.org.



Externally-Led Patient Focusd Drug Development Meeting

Join NAF and CureDRPLA on September 25th for a virtual Externally-Led Patient Focused Drug Development (EL-PFDD) meeting. The meeting kicks off at 10 AM Eastern Time via a live stream on YouTube. This interactive meeting will focus on the Polyglutamine Spinocerebellar Ataxias (SCA1, SCA2, SCA3, SCA6, SCA7, SCA17, and DRPLA). Throughout this meeting, there will be opportunities to: participate in live polling questions, call in to provide comments, submit written comments, and hear from patients and caregivers.

If you would like to submit written comments ahead of time, please indicate your SCA type and name and email comments to: **comments@ataxia.org**.



Friday, September 25, 2020
10:00am - 3:00pm EDT

(7am- 12pm PDT, 8am-1pm MDT, 9am-2pm CDT)

Mark your calendars now! Registration is unlimited and will open the morning of September 25th on the NAF website.

Please Join Us!

From the comfort of your home anywhere in the world you can attend this important virtual meeting for the future of Ataxia treatments.

More information or to view our informational webinar to learn more about what this meeting means, how the process will work and how you can get involved visit:
https://ataxia.org/el-pfdd-meeting/

NAF
National Ataxia Foundation
www.ataxia.org

CURE DRPLA

WALK N'ROLL TO CURE ATAXIA

National Ataxia Foundation

NAF Virtual Walk N' Roll
Saturday, October 3, 2020

1:00pm EDT | 12:00pm CDT
11:00am MDT | 10:00 PDT

Register for free to join us and win prizes. Participate in a virtual silent auction. During this 90 minute live-streamed event presented by Cadent Therapeutics and Biohaven Pharmaceuticals, you will hear from leading Ataxia researcher Dr. Chip Wilmot, learn about an Ataxian looking to break a unique Guinness Book of World records and so much more!

For more information and to register visit:
www.ataxia.org/2020-walk-n-roll-goes-virtual/

Thanks to our Sponsors

biohaven pharmaceuticals

Cadent Therapeutics

HOW DOES A CLINICAL TRIAL WORK?

Amber Trzeciak

Clinical trial phases can be a confusing concept to understand. The purpose of clinical trials is to determine how a drug or device (intervention) will interact with a human being. Trials are regulated by our governmental agency the FDA (US Food and Drug Administration). They set forth many guidelines and rules that sponsors (those testing the intervention) must follow before administering their drug or device into human volunteers. Before getting into clinical trial phases, first we will address how a drug or device gets to the point of being approved for administration to humans.

Non-Clinical Development Phases

Every intervention idea begins in a laboratory. This is where researchers begin to develop and discover products or compounds that will help stop or reverse the effects of a disease or condition. From this development phase, the device or compound moves into preclinical research. Here researchers will test their device or compound in vitro, which is in cells in dishes or vials, or in vivo, which is in living animals (rodents, dogs, monkeys, etc). The purpose of preclinical work focuses heavily on determining the amount of drug or device that is considered safe and not toxic.

Once the researchers determine that they have a device or drug compound that they think will work they submit an application to the FDA. This application is called an Investigational New Drug (IND) and it lays out the specifics of what a drug developer (sponsor) hopes that their device or compound will accomplish. The application also includes details of all the current research on this device or compound, including the demonstrated safety and tolerability of it. Most importantly, it includes a protocol. A protocol is a study design plan that maps out what the clinical trial will look like. For example, the protocol will provide information on duration of the trial, who can and cannot participate in the trial, how the device or drug will be administered, and any assessments to be included (for example, blood draws). If the IND is approved by the FDA, the sponsor can move into starting their clinical research.

Clinical Development Phases

Clinical research starts when a drug compound or device is granted access to begin its clinical trial phases. Clinical trials can have any where from 0-4 phases, with phases 1, 2, and 3 being the most common.

Phase 1 Clinical Trial

Most sponsors start with a Phase 1 clinical trial. A Phase 1 trial most often uses healthy volunteers who do not have the disease or condition in which the drug or device may treat. A Phase 1 trial checks for safety and tolerability of the drug, or more simply how the drug affects the body and how it should be given (for example, tablet versus capsule). Most importantly, a Phase 1 trial monitors for any potential side effects. If a Phase 1 trial is determined to be successful it moves onto the next trial phase, Phase 2.

Phase 0 Clinical Trial

At times, there may be mention of a Phase 0 clinical trial, this is less common, but often involves very small amounts of volunteers who have the disease or condition that is hoping to be treated. Often these volunteers participate for a much shorter time and are given a smaller dose of the drug compound. The purpose of a Phase 0 trial is to help expedite moving on through the other phases, or more simply put, weeding out the bad compounds early on without wasting time and resources.

Phase 2 Clinical Trial

A Phase 2 clinical trial involves actual patients who are impacted by the disease or condition that the drug compound or device is hoping to target. The intent of a Phase 2 trial is to test the "efficacy", which is whether the drug does what the researchers intended, and if it causes any side effects. The volunteer population is usually larger than the first phase trial and the duration can vary depending on the sponsor's needs. There are different options available for a Phase 2 trial that can include what is called "open-label" or "placebo-controlled". The difference is that if a trial is open label, everyone in that

trial receives the actual drug, versus a placebo-controlled trial that offers some volunteers a placebo intervention (non-drug) whereas some volunteers are given the actual drug. If this route is chosen, a sponsor can also determine to make the trial blinded (no one knows who receives actual drug or placebo) or unblinded (the sponsor knows who received the actual drug versus the placebo intervention). The take-away for going with a blinded study is that your results will more likely be accurate due to the elimination of any bias. If the Phase 2 trial is determined to be successful, it is approved to move onto the next trial phase, Phase 3.

Phase 3 Clinical Trial

The purpose of a Phase 3 clinical trial is to determine if a drug or device offers treatment for a specific disease or condition within a widespread population safely. The main information gained from a Phase 3 is most importantly, safety data, which determines if there any long-term side effects. Typically, this trial phase includes the largest number of participants and lasts the longest. To be considered successful in this phase, the drug or device must be considered safe and prove to be more effective or as effective as other treatments available.

Once a Phase 3 trial begins to wrap up and show positive data, the sponsor seeks approval of this drug or device from the FDA. If the drug or device is filling an unmet need, the team can request to be fast tracked by the FDA which allows for priority review from the FDA. This means that the FDA must review the data and information within 6 months of receiving the application. Another benefit to being considered fast track by the FDA is that the drug or device can be eligible for accelerated approval. The FDA can then grant authorization of this drug or device based upon evidence presented. These designations are only for drugs or devices that provide a new or better treatment option for a disease or condition.

Phase 4 Clinical Trial

A Phase 4 clinical trial occurs after a drug or device has been approved by the FDA and can be used by patients. It continues to monitor the safety and efficacy of that intervention, in real time. This trial occurs without the conditions of being conducted in a research facility and can provide feedback on details that may have not otherwise been noticed.

Why is it important to participate in a clinical trial?

Without volunteers, many medications and devices would not be where they are today. We thank you for your participation!

How to find clinical trials that may be relevant to you:
First and foremost, the FDA has created an online portal where you can search for clinical trials by disease, condition, location, phase and by sponsor. **www.clinicaltrials.gov**

Another alternative is to use the Pipeline page, found on most sponsor's websites, for example: **http://www.cadenttx.com/pipeline/**

These pipelines provide a quick snapshot of where the company is in the process of the clinical trial phases for their specific disease or condition. Resources: FDA (**https://www.fda.gov/patients/learn-about-drug-and-device-approvals**)

Amber Trzeciak , Cadent Therapeutics, Inc., Patient Partnership and Advocacy Manager
Amber Trzeciak is the Patient Partnership and Advocacy Manager for Cadent Therapeutics in Cambridge, Massachusetts. She joined the small biotech company in 2017. Originally from Pittsburgh, PA, Amber transitioned to the Alexandria, VA area during her 10 plus years as a Mental Health Counselor. Amber brings nearly 20 years of advocacy experience to her work within the biotechnology field. She has a passion for helping others and often finds herself rallying friends, family, and colleagues to help at events that she supports.

Amber has a M.S. in Clinical Mental Health Counseling from Indiana University of Pennsylvania, a M.S. in Legal Studies-Law and Public Policy Concentration from California University of Pennsylvania and her bachelor's degree is in Criminology, also from Indiana University of Pennsylvania.

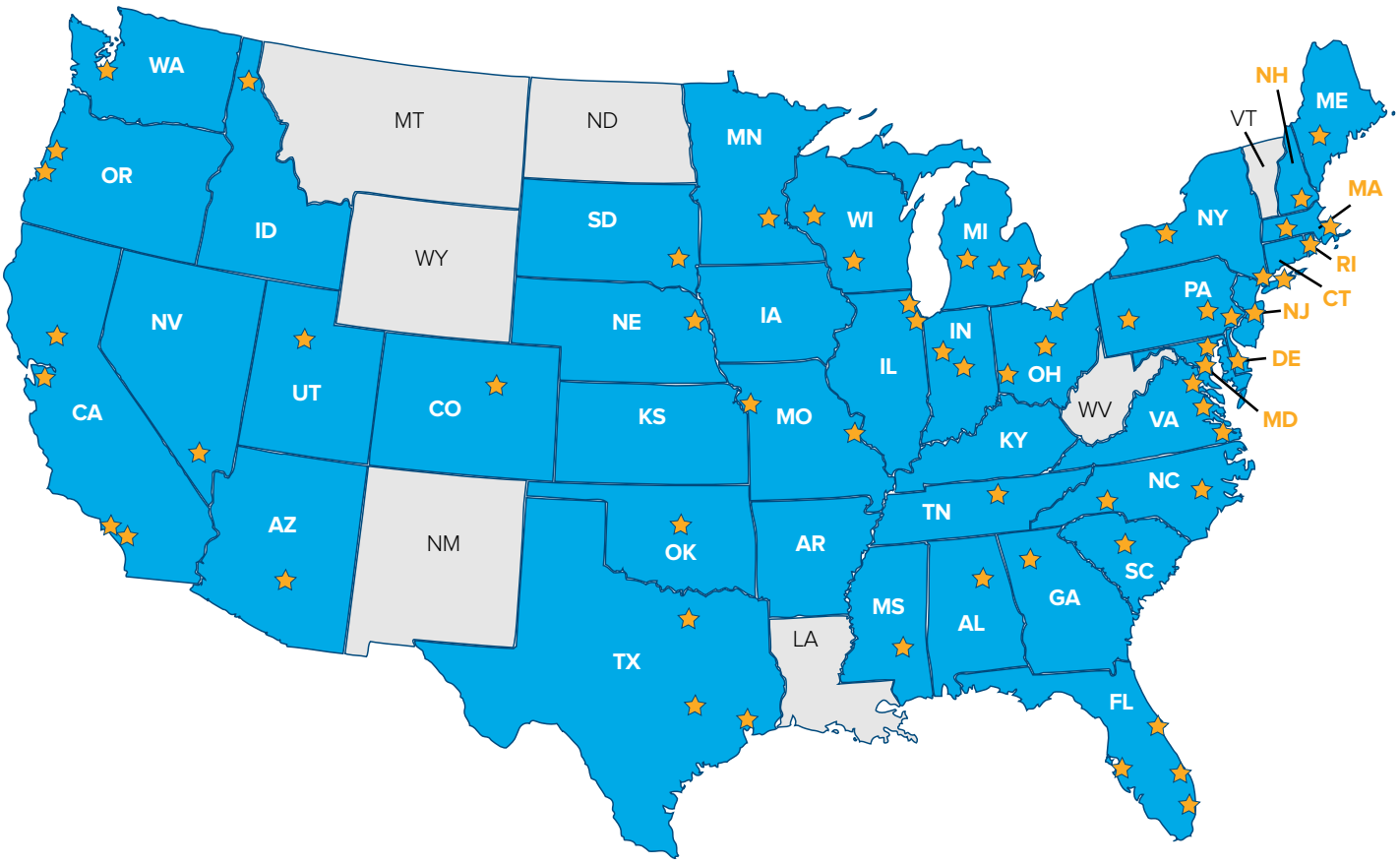
Support Groups

The most current support group and event information is available on the NAF website, www.ataxia.org.

Support groups can remind us that we are not alone. Each person experiences Ataxia in a different way, but we still have many things in common. A benefit of attending a support group is having the chance to talk with others and learn how different people deal with the same disease.

If you or a loved one has been newly diagnosed with Ataxia, please contact the support group leader nearest you. If there is not a group in your area, we encourage you to visit our online social networks. You may also consider starting a support group in your area or becoming an NAF ambassador. If you are interested in these volunteer positions please contact Lori Shogren of the NAF staff at lori@ataxia.org or (763) 231-2743.

Come. Learn. Share. But most of all, know that you are NOT alone.



Join one of our Facebook Support Groups!

NAF Support Group	*www.facebook.com/groups/NAFmail*
Under 30 with Ataxia	*www.facebook.com/groups/under30withataxia*
Parents of Ataxia	*www.facebook.com/groups/ParentsOfKidsWithAtaxia*

Support Group News and Community Events

https://ataxia.org/category/support-groups/support-group-news/

New Ataxia Support Group

Spouses and Partners of Loved Ones with Ataxia

This group is for spouses and partners without Ataxia who have a spouse or partner with Ataxia. This is our spot to learn from one another, vent, have fun, and discuss topics important to us!

Dana Mauro, Support Group Leader
E-mail: ataxiafacts@gmail.com
Facebook Group: https://www.facebook.com/groups/AtaxiaSpousesAndPartners/

Read the Latest Support Group News!

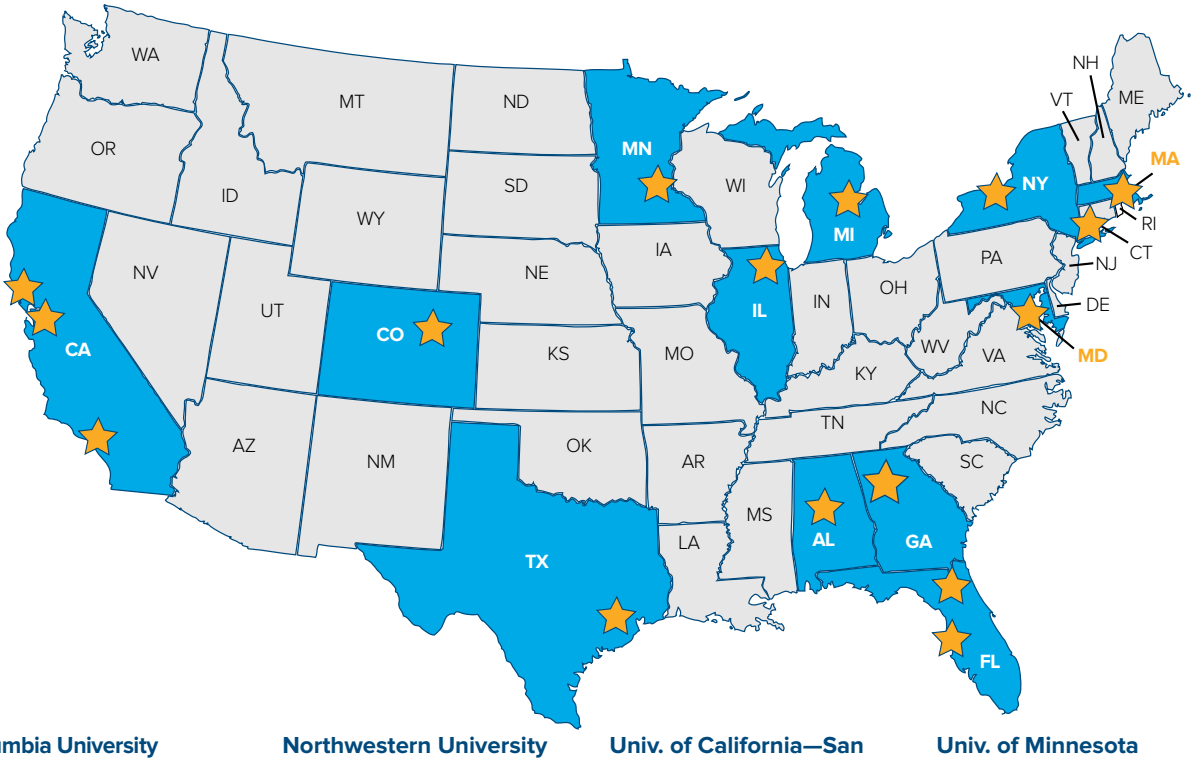
Interested in learning about what's going on at local support groups? Check it out at www.ataxia.org/SupportGroupNews

COVID-19 and Support Group Update

In a continued effort to protect our Ataxia community during the COVID-19 pandemic, with the health and safety of the Ataxia community in mind, and after continued consultation with our Medical and Research Advisory Board (MRAB), NAF has made the decision to extend our recommendation to support groups to cease all in-person events and meetings for the remainder of 2020. Many of our leaders have moved to virtual platforms, such as video chats and conference call services. We hope to see you join a virtual meeting and say hello!

READISCA Observational Study for SCA 1 & 3

Looking for participants with SCA1 and SCA3 to help with the READISCA study at any of the sites across the US. One of the main goals of this study is to get ready for treatment trials. READISCA does not involve medication or treatment therapy, but it will collect crucial data that will help future clinical trials. The main goals of this study are to establish the world's largest group of early stage and symptomless SCA1 and SCA3 individuals.



Columbia University
New York, NY

Emory University
Atlanta, GA

Houston Methodist Hospital
Houston, TX

Johns Hopkins University
Baltimore, MD

Mass General Hospital (Harvard)
Boston, MA

Northwestern University
Chicago, IL

Stanford University
Palo Alto, CA

Univ. of Alabama—Birmingham
Birmingham, AL

Univ. of California—Los Angeles
Los Angeles, CA

Univ. of California—San Francisco
San Francisco, CA

Univ. of Chicago
Chicago, IL

Univ. of Colorado—Denver
Denver, CO

Univ. of Florida
Gainesville, FL

Univ. of Michigan
Ann Arbor, MI

Univ. of Minnesota
Minneapolis, MN

Univ. of Rochester
Rochester, NY

Univ. of South Florida
Tampa, FL

★ Indicates location of Clinical Trial Site

For more information on READISCA contact:

Houston Methodist Research Institute

Tetsuo Ashizawa, MD—Contact PI/PD

Phone: 346-238-5021 • Email:U01SCA1&3@houstonmethodist.org

Memorials and In Your Honor

NAF is grateful to those who have made contributions in memory of or in honor of their friends and families whose names are listed below. This list reflects contributions made in December 2019 - March 2020.

In Honor

Amber Bratt
Anne Powell
Ashleigh Thiele
Bent Family
Brandon Barker
Brett Masserant
Candice Matykowski
Carmen Pieragastini
Carol Stabenow
Cathy Bethay
Celia Baculi
Chanda Huy
Chicago Ataxia Support Group
David Murphy
Debra Covington
Derringer Kuriatnyk
Destinee Juarez
Dianne Jones
Dirk Desserault
Donald Santa Croce
Dr. Jeremy Schmahmann
Edward Schwartz
Elissa Fisher
Ellen Moetsch
Fleming Family
Francine Zdrale
Great Atlanta Ataxia Support Group
Greg Rooks
Harvey Blanton Ray
Ibtissam Bahmane
Irene Costa
Janet Kepple
Jeannie Smith Ball
Jeremy Hume
Jessica Budreau
Jim Devlin
Joe Sweeney
Jonathan Zilles
Jose Alfredo Moran
Judy Johnson
Julie Quinlivan
Karen Suchomel
Kathy Hoffman Bucholz
Kelly and Kim Tambourino
Kent Hardel
Kevin Zwick
Kim Poor
Kory Macy
Kyin K. Fong
Lani Bassett
Lindsey Graham Guinn
Lisa A Cole
Lisa Cole
Loren Jones
Lori Hoffman Goetzman
Louise Estabrook
Marc Alessi
Mary Fuchs
Mary Hartmann
Mary Schlickbernd
Matt Pickering
Matt Stabenow
Michael Rooth
Mike Cammer
Mike Derosa
Nan Vail
Nate Stabenow
Nathan Benes
Olivia Schumacher
Pam Perault
Paul Dolan
Penni Sutherland
Raymond Robinson
Rick Roemke
Rita Garcia
Robb Lubin
Ryan Mortier
Scott Schloesser
Scott Tabor
Sgt. Colin Guertin
Steve Ofenstein
Steven Brune
Sudha Yarraguntla
Tanya Goldman
The Demint Family
The Hinman Family
The Sancho Family
The Scruggs Family
Tom Sweeney
Wayne Walters
Wiese Family
Zachary Zmithrovitch
Zack Stackle

In Memorial

Alex Atzeff
Annie Gulliver
Antoinette Varron
Arthur Stackle
Aymee G. Torres, Dr. Aymee A. Torres -Michels, Ricardo Luis Guerrero & Teresita Guerrero
Ben Dowling
Betty Evans
Bill Bassett
Bill Ellis
Brittany Mumford
Carol Greenblatt
Carolyn Simmons
Catherine O'Brien Edge
Charles C. Williams
Cheri Morse
Chuck Mitchell
Clair and Betty Beck
Claire Martin

In Memorial Cont.

Clete and Peggy Brunnert
Cletus Brunnert
Dean Bastion
Debi Adair
Dianne Siegel
Don Antonellis
Donna Kannal
Doris Ruse
Esther Cowart
Eugene Marshall
Fola Odegbami
Frank Pellegrino
Gary Hartsock
Gary Peterson
Greg Rest
Handoyo Triputra
Harold Pfeifer
Hazel Doty
Hugo Quintanilla
Jacqueline Guercio
Jacqueline Williams
Jame Edward Tilley
James Keating, III
James Teaney
Jane Haley
Janet Coyne and Stacy Coyne Leger
Jeannie Marie Teague
Jeff Crowe
Jeff Suhr
Joanne Denney
Joao and Silvana Martinho
John Brennan, Jr.
Jordan and Sydney Hubbard
Joseph Christie, Sr.
Joseph Gaither, III
Joseph Martinho
Joyce Kennedy
Judith Ann Jehnke
Judy Jehnke
Karen Brueske
Kathryn D Smithers
Katie Gulliver
Ken Loya
Kenneth Clark
Kevin Michael Fleming
Krista Humes
Kristie Samuels
Lynda Gillam
Marcia Ritschel
Maria Amelia Viveiros
Marilyn Hazel
Mary Alice Hopkins
Michelle Parent
Mike Rooth
Murielle Paquin Arrol
Nancy Barbeau
Navidad Sarmiento
Nestor Delgado
Pam Wollangur
Pansy Gooch
Patricia Elsom
Pete Ressel
Peter Harris
R.W. King
Raymond Roderick
Richard Madris
Richard Weber
Robert Dugal
Robert Tabor
Ronald Randol
Schoenrogge Family
Serena Garcia
Sharon Baggett
Silvana Martinho
Smudge Taylor
Stephanie Bergquist
Susan Madras
Thora Mae Lankton
Tony Ruse Hanson
Vicki Winston
Vince Dadd
Walter Herbert Jones
Wilma Stackle

BRAIN TISSUE DONATION PROGRAM

Ataxia researchers have made many discoveries because of donations of brain tissue from those affected with Ataxia. NAF's Brain Donation Program was established to allow those who desire to donate their brain upon death so that researchers can find more answers.

If you are interested in learning more about brain donation, you may contact Mary Ann Peterson, NAF Research Assistant, at ***mary@ataxia.org*** or **763-231-2750**.

Natural History Study needs SCA Research Participants

The Clinical Research Consortium for the Study of Cerebellar Ataxia (CRC-SCA) continues to recruit research participants who have a confirmed diagnosis of SCA 1, 2, 3, 6, 7, 8 or 10. This is an opportunity for anyone in the United States with those forms of SCA at any stage of the disease to participate. Contact the research coordinator at a site near you to learn more about how you might be able to help in Ataxia research efforts to discover a treatment.

The National Ataxia Foundation encourages anyone with SCAs 1, 2, 3, 6, 7, 8 and 10 to participate.

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Houston Methodist
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Johns Hopkins University
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University of California San Francisco
Julia Glueck
Julia.Glueck@ucsf.edu

This research is generously supported by the Gordon and Marilyn Macklin Foundation and the National Ataxia Foundation.

For more information on the study, you may contact Sue Hagen at susan@ataxia.org or 763-231-2742

Disappointed that you don't qualify for this research study?

Take an important step to make sure that you are notified of future studies for which you might qualify. Enroll in the CoRDS Ataxia Patient Registry.

If you are affected with any type of Ataxia, enroll in the registry by going to the website: https://cordsconnect.sanfordresearch.org/BayaPES/sf/screeningForm?id=SFSFL. If you have questions about enrollment in the registry, contact the CoRDS staff at 877-658-9192.

NAF Staff Directory and Social Networks

NAF Staff Directory:

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Kelsey Trace, Research Manager	(763) 231-2742 • *kelsey@ataxia.org*

Social Networks:

NAF Facebook Page	*www.facebook.com/ataxiafoundation/*
NAF YouTube Channel	*www.youtube.com/user/NatlAtaxiaFound*
NAF Twitter	*www.twitter.com/NAF_Ataxia*
NAF LinkedIn	*www.linkedin.com/company/nationalataxiafoundation*

THE ATAXIA COMMUNITY IS INTERESTED IN YOUR GREAT IDEAS.

If you have Ataxia Tips or a personal story you would like to share in a future issue of *Generations*, please submit it to ***naf@ataxia.org***. Those submitting a personal story are asked to please include a photo or two and a brief author bio (1-2 sentences).

THE DEADLINE FOR SUBMITTING MATERIALS

for the Fall issue of *Generations* is September 10, 2020. Please send articles, your personal story, recaps of Ataxia-related events, photos and reports to ***naf@ataxia.org***. Thank you.

YOUR COMMUNITY NEEDS YOU!

-Do you have an Ataxia tip?

-A story about your Ataxia journey?

NAF needs your help. We are collecting Ataxia tips and stories to share in future editions of *Generations*.

Your tip or story just might be the help someone else is looking for.

Submit your tips and stories today!
send to: *naf@ataxia.org*

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Minneapolis, MN 55426
(763) 553-0020

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