National Ataxia Foundation
58th Annual Membership Meeting

Mike Parent, Executive Director

Welcome to Denver

Disclaimer

- The information provided by speakers in any presentation made as part of the 2015 NAF Annual Membership Meeting is for informational use only.
- NAF encourages all attendees to consult with their primary care provider, neurologist, or other health care provider about any advice, exercise, therapies, medication, treatment, nutritional supplement, or regimen that may have been mentioned as part of any presentation.
- Products or services mentioned during these presentations does not imply endorsement by NAF.

Presenter Disclosures

- No relationships to disclose or list

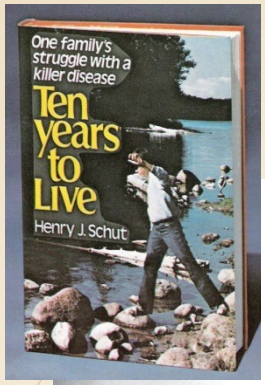
National Ataxia Foundation

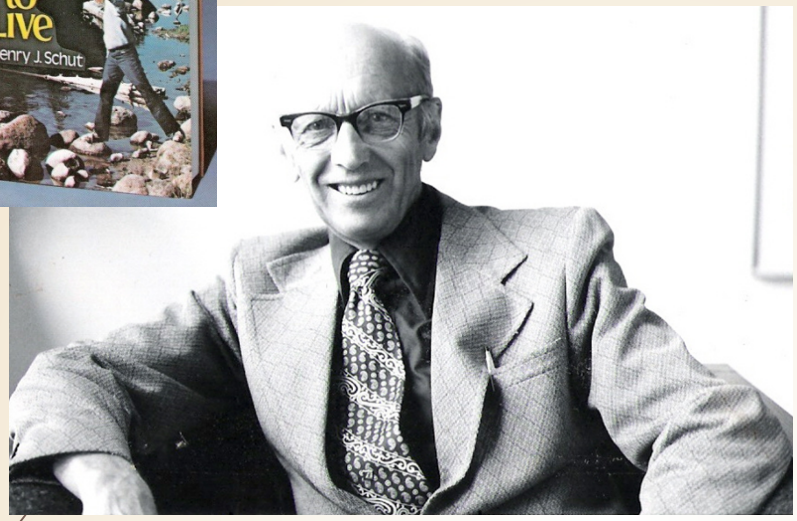
The National Ataxia Foundation was formed in 1957 by two brothers , Dr. John W. Schut and Henry J. Schut and along with other family members and friends.

The Founders:

Dr. John W Schut, a brilliant physician and researcher, spent a major part of his life trying to find a cure for ataxia, which eventually claimed his life. He was instrumental in starting the National Ataxia Foundation which continues to carry on the research he started.

National Ataxia Foundation





Henry J. Schut was a member of the NAF Executive Committee and former President of the National Ataxia Foundation. Mr. Schut was a math instructor at Maple Lake High School in Minnesota and dedicated his life in creating awareness about ataxia. In 1978, Henry Schut published a book called "Ten Years to Live," a story about his family's struggles with ataxia. The book has sold world-wide.

National Ataxia Foundation

The ***Main Focus*** of the Foundation in the 1950s was to help foster ataxia research and to find families affected by ataxia

The 1st annual membership meeting was held in 1958 in Minnesota. Nine members attended.

National Ataxia Foundation

Today, the annual membership meeting averages between 400-650 attendees

National Ataxia Foundation

Who is the National Ataxia Foundation(NAF)?

4. The NAF is a membership supported nonprofit organization established in 1957 and is dedicated to serving those with ataxia and their families. In fact, when the NAF first began, a membership was only given after the application was reviewed at the annual membership meeting. It was not until the 1970's when open memberships were accepted.

National Ataxia Foundation

What is the Legal Structure of the NAF?

- The NAF was incorporated under the laws of Minnesota as a nonprofit corporation. It is recognized by the IRS as a 501(c)(3) corporation. The NAF is overseen by a Board of Directors who are elected by the membership.

What is the Primary Focus of the NAF?

- The mission statement of the NAF is: "*The National Ataxia Foundation is dedicated to improving the lives of those affected by ataxia through support, education and research.*"

National Ataxia Foundation

When did the NAF Begin Direct Funding of Ataxia Research?

- The NAF first ataxia research grant was awarded to Dr. Robert Currier from the University of Mississippi in 1978 in the amount of $5,000. The next year NAF funded two research studies totaling $5,750. Over the past 16 years the NAF has awarded funding to 240 ataxia research studies in 14 countries. Over the past four years, nearly four million dollars in research has been awarded.

Dr. Robert Currier and his daughter Dr. Mary Currier 1983

National Ataxia Foundation

What Funding Sources Help Support the NAF's Programs?

Chapters/Support Groups
Combined Federal Campaign
Corporate Support
Deferred Giving/Estates
Employer Matching Gifts
Endowment Funds
Family Fund Raisers

Foundation Support
Fund Raising Events
Gifts of Stock/Assets
Group/Family/Individuals
IAAD EVENTS
Membership Support
Memorials/In Honor of
Partnerships

Planned Giving
Recurring Gifts
Research Drive
Social Networking
Special Projects
United Way
Vehicle Donations
Walk n' Rolls

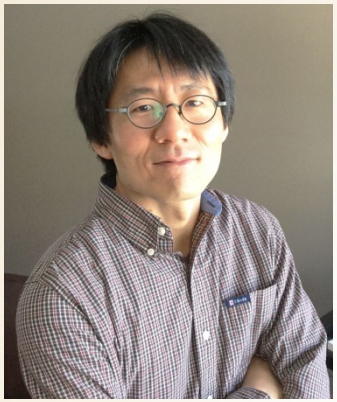
National Ataxia Foundation

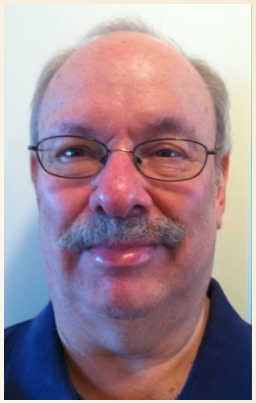
From the Funds Raised, how are the Funds Used ?

4. While the 2014 annual audited financial statements will be soon available, the 2013 CPA annual audited financial statements reflects fundraising costs were 4.5% of total expenditures and administrative costs were 5.3% of total expenditures. Program Services for 2013 totaled 90.2% of total expenditures, in other words, 90.2 cents out of every dollar spent was used to support research and programs. In the Program Services area, the highest expenditure was research, totaling 64% of all expenditures for fiscal year 2013.

Current Programs

Fostering Research:

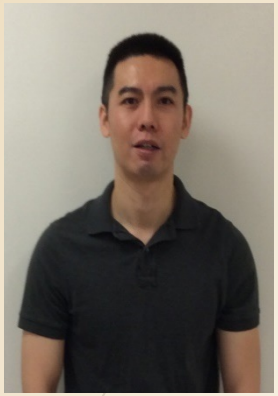





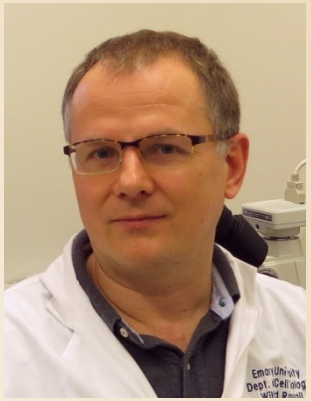




National Ataxia Foundation

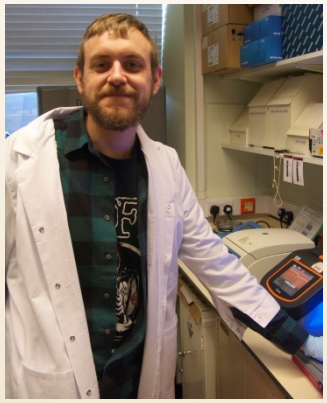
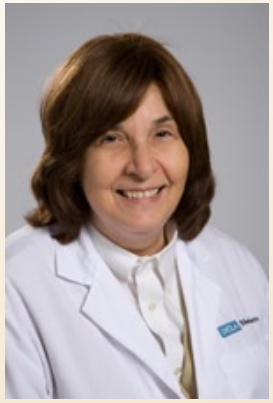










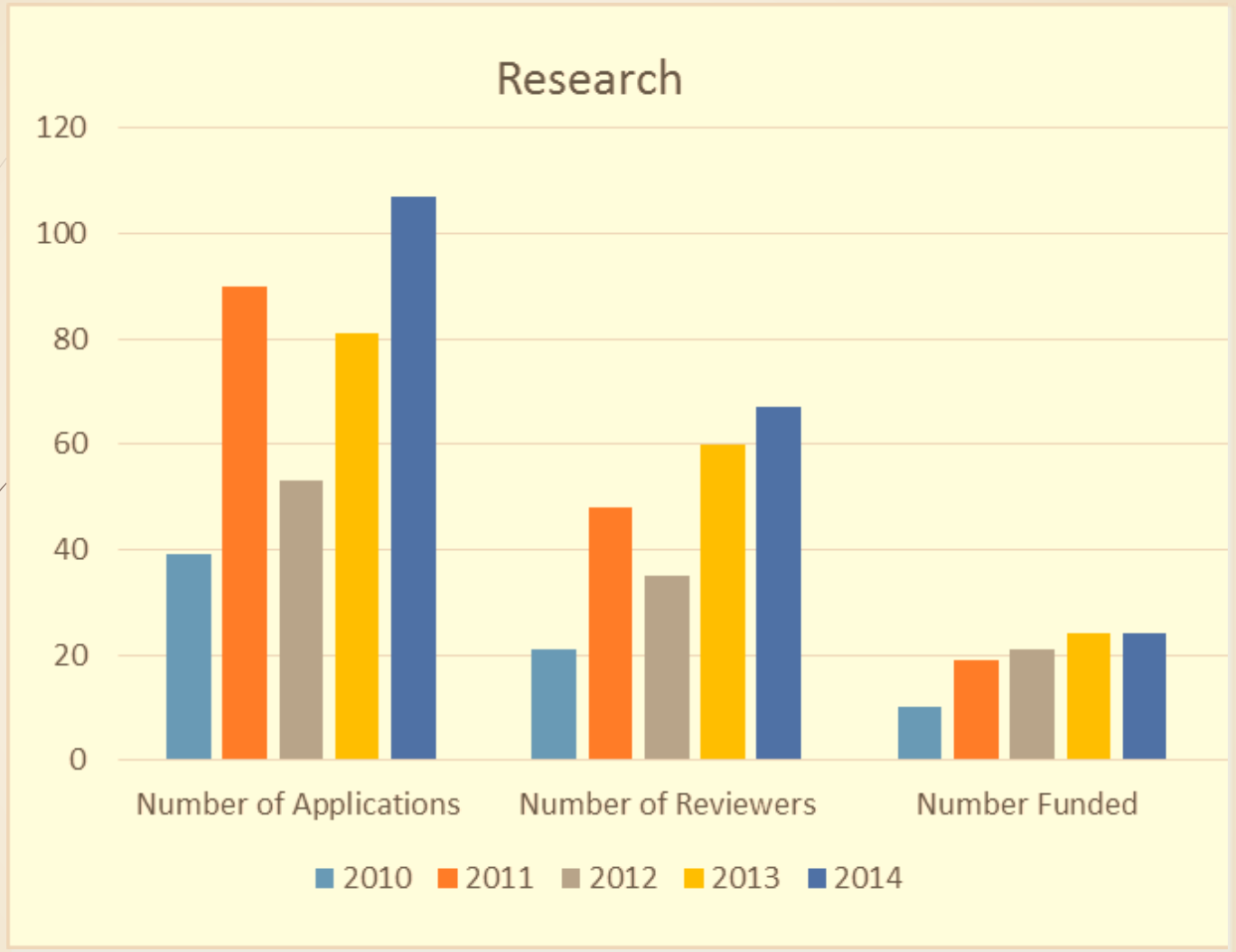

Direct Research Funding:

4. The Theme for this year's research drive was **Research…Discoveries…Answers**.

In supporting these efforts, the NAF has established five vital ataxia research programs to provide direct funding for basic science and translational studies to find treatments and a cure for hereditary and sporadic ataxia.

In 2014 there were 68 peer reviewers assigned to evaluate 107 research applications from 19 countries, 226 individual reviews were given. The largest number of research applications and highest number of reviews in the NAF's history.

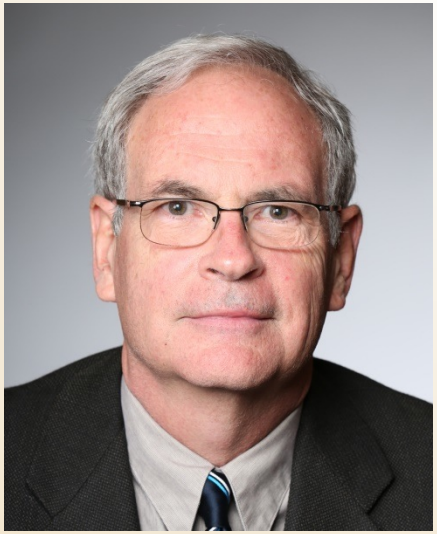
In 2014, the NAF supported 24 ataxia research studies through donations and partners totaling nearly 1 million dollars in ataxia research, including a new partnership with the American Academy of Neurology's American Brain Foundation to fund a two-year Clinical Research Training Fellowship in Ataxia.



5 Year Graph of the NAF Research Activities (2010-2014)

A Special Thank You:

Dr. Harry T. Orr, the NAF's Research Director and Dr. Laura Ranum, the NAF's Associate Research Director lead up the NAF 2014 research review process. A special thank you to Drs. Orr and Ranum in helping to insure that the best science in the world was funded.



Harry T. Orr, PhD

Laura Ranum, PhD

National Ataxia Foundation

CoRDS Registry

Coordination of Rare Diseases at Sanford

CoRDS–NAF Patient Registry:

an International Disease-specific Registry for Individuals with Ataxia

SANFORD RESEARCH

What is the CoRDS-NAF registry?

- Collaboration between CoRDS and the NAF to create a registry that collects and organizes information from individuals with Ataxia (established 2013)
- Researchers are able to review **de-identified** data
- Participants may be notified of research studies for which they are eligible

SANFORD RESEARCH

CoRDS Team

David Pearce, PhD
Principal Investigator

Catie Olson
Director

Austin Letcher
Sr. Research Associate

- CoRDS Scientific Advisory Board
- Sanford Research Institutional Review Board (IRB)
- Sanford Health Research Information Technologies (RIT)
- Sanford Health Information Technologies (IT)
- Sanford Health Marketing & Digital Strategies
- Sanford Health Foundation

SANFORD RESEARCH

CoRDS-NAF Registry Metrics

- The CoRDS-NAF registry includes:
  - 739 fully enrolled participants
  - 544 participants in screening

Participants have enrolled from

- 49 US States
- 18 Countries

SANFORD RESEARCH

How are patients and families involved?

- Participants that enroll can contribute information to the CoRDS-NAF registry by filling out a brief questionnaire
- Enrollment involves reading the consent form and completing the questionnaire
- Participants may learn about research opportunities for which they are eligible
- When notified about research opportunities, you have the ability to decide whether or not to participate

SANFORD RESEARCH

Why would an individual want to participate?

- Provides participants an opportunity to be informed of research studies and clinical trials for which they are eligible
- Provides researchers with a central resource for the identification and more rapid recruitment of potential research participants
- Has the potential to accelerate research into rare diseases
- No financial cost with easy and quick enrollment

SANFORD RESEARCH

In-person Enrollment

- Informed Consent form and CoRDS-NAF questionnaire available for pick-up
- Read the Informed Consent & complete the questionnaire and return to CoRDS table
- The questionnaire is brief and takes about 30 minutes to complete

SANFORD RESEARCH

Enrollment Options

1. Online enrollment on the CoRDS website
2. Mail-based enrollment
3. In-person enrollment

SANFORD RESEARCH

Don’t forget!

If you filled out a screening form, but haven’t completed your questionnaire, you aren’t fully enrolled. Speak to a CoRDS team member about finishing enrollment today!

SANFORD RESEARCH

The NAF's
5th Ataxia Investigators Meeting
(AIM 2014)

This scientific meeting on ataxia was attended by more than 150 world-leading ataxia clinicians, scientists, patent advocacy and industry representatives from 16 countries to create a forum to help accelerate world-wide ataxia research. Senior and junior investigators met in 2014 to achieve four primary goals:

1. Identify common disease mechanisms
2. Explore therapeutic strategies
3. Help establish the future leaders of ataxia research
4. Bring trainees (graduate students and postdocs) into contact with people with ataxia and their families

In addition, the NAF continues to partner and support various scientific conferences which furthers ataxia research.

International Ataxia Awareness Day (IAAD) September 25

National Ataxia Foundation


National Ataxia Foundation
LOU COLETTI
MEMORIAL TOURNAMENT


Central Massachusetts Support Group
Raising Awareness and Funds to Fight Ataxia
National Ataxia Foundation


3663
3661
2212
3384
5701


Walk n' Roll for Ataxia
National Ataxia Foundation
Greater Atlanta Ataxia Support Group


The International Ataxia Awareness Day Trophy Race
Divine Excitement
"Maiden"
Brereton C. Jones, owner
J. Larry Jones, trainer
5 1/2 Furlongs - 1:06
Delaware Park 9/25/14
Kerwin C. Clark, up
2nd Jane Says
3rd Latest Thinking
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INTERNATIONAL ATAXIA AWARENESS DAY

Each one of us can make a difference in getting the word out about ataxia. Dana Mauro from Massachusetts is a great example of this:

Another awareness initiative for 2014 was the introduction of the Ataxia Presentation –PowerPoint

This ataxia awareness tool allows groups and individuals to provide a greater understanding about ataxia in their local communities.

You c 

website at

In 2014, four PSAs on ataxia were released for distribution to local media. Here is one example:

National Ataxia Foundation

Social Media:

The NAF will continue to strengthen our connections with the ataxia community by participating in social media platforms.

	2014	2013
NAF Bulletin Board	68,388 Members	61,949 Members
	9 Forum Discussions	11 Forum Discussions
	2,202 Topics Posted	2,717 Topics Posted
NAF Facebook Group	4,443 Members	3,636 Members
NAF Facebook Page	3,137 Likes	2,493 Likes
NAF Twitter Page	886 Follows	674 Follows
	951 Tweets	755 Tweets
NAF YouTube Channel	98 Subscribers	49 Subscribers
	8,127 Video Views	849 Video Views

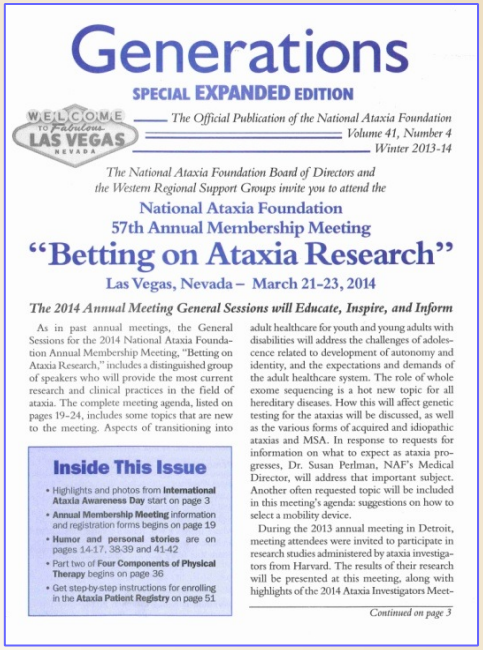
National Ataxia Foundation

Annual Membership Meeting 2014

The 57th National Ataxia Foundation's Annual Membership Meeting was held in Las Vegas, NV in March. Hosted by the Western Region which includes the states of Alaska, Arizona, California, Hawaii, Idaho, Nevada, Oregon, Utah and Washington.

Nearly 650 attended the three-day conference. Attendees came from 41 US states, Washington DC and from 9 additional countries including Australia, Canada, Denmark, France, Germany, Ireland, Japan, Mexico and the United Kingdom.

The National Ataxia Foundation now offers more than 20 *Frequently Asked Questions* (FAQs) Fact Sheets as well as the quarterly newsletter *Generations*.
In addition, the NAF offers books and other publications on ataxia.



Generations

SPECIAL EXPANDED EDITION

WELCOME to Fabulous LAS VEGAS NEVADA

The Official Publication of the National Ataxia Foundation
Volume 41, Number 4
Winter 2013-14

The National Ataxia Foundation Board of Directors and the Western Regional Support Groups invite you to attend the

National Ataxia Foundation
57th Annual Membership Meeting

"Betting on Ataxia Research"

Las Vegas, Nevada – March 21-23, 2014

The 2014 Annual Meeting General Sessions will Educate, Inspire, and Inform

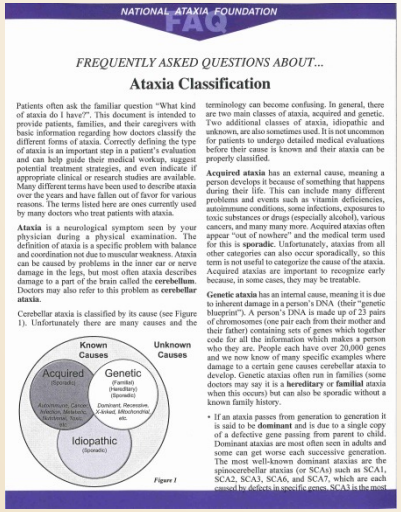
As in past annual meetings, the General Sessions for the 2014 National Ataxia Foundation Annual Membership Meeting, "Betting on Ataxia Research," includes a distinguished group of speakers who will provide the most current research and clinical practices in the field of ataxia. The complete meeting agenda, listed on pages 19-24, includes some topics that are new to the meeting. Aspects of transitioning into adult healthcare for youth and young adults with disabilities will address the challenges of adolescence related to development of autonomy and identity, and the expectations and demands of the adult healthcare system. The role of whole exome sequencing is a hot new topic for all hereditary diseases. How this will affect genetic testing for the ataxias will be discussed, as well as the various forms of acquired and idiopathic ataxias and MSA. In response to requests for information on what to expect as ataxia progresses, Dr. Susan Perlman, NAF's Medical Director, will address that important subject. Another often requested topic will be included in this meeting's agenda: suggestions on how to select a mobility device.

During the 2013 annual meeting in Detroit, meeting attendees were invited to participate in research studies administered by ataxia investigators from Harvard. The results of their research will be presented at this meeting, along with highlights of the 2014 Ataxia Investigators Meet-

Continued on page 3

Inside This Issue

- Highlights and photos from **International Ataxia Awareness Day** start on page 3
- **Annual Membership Meeting** information and registration forms begins on page 19
- **Humor and personal stories** are on pages 14-17, 38-39 and 41-42
- Part two of **Four Components of Physical Therapy** begins on page 36
- Get step-by-step instructions for enrolling in the **Ataxia Patient Registry** on page 51



NATIONAL ATAXIA FOUNDATION

FAQ

FREQUENTLY ASKED QUESTIONS ABOUT...

Ataxia Classification

Patients often ask the familiar question "What kind of ataxia do I have?". This document is intended to provide patients, families, and their caregivers with basic information regarding how doctors classify the different forms of ataxia. Correctly defining the type of ataxia is an important step in a patient's evaluation and can help guide their medical workup, suggest potential treatment strategies, and even indicate if appropriate clinical or research studies are available. Many different terms have been used to describe ataxia over the years and have fallen out of favor for various reasons. The terms listed here are ones currently used by many doctors who treat patients with ataxia.

Ataxia is a neurological symptom seen by your physician during a physical examination. The definition of ataxia is a specific problem with balance and coordination not due to muscular weakness. Ataxia can be caused by problems in the inner ear or nerve damage in the legs, but most often ataxia describes damage to a part of the brain called the **cerebellum**. Doctors may also refer to this problem as **cerebellar ataxia**.

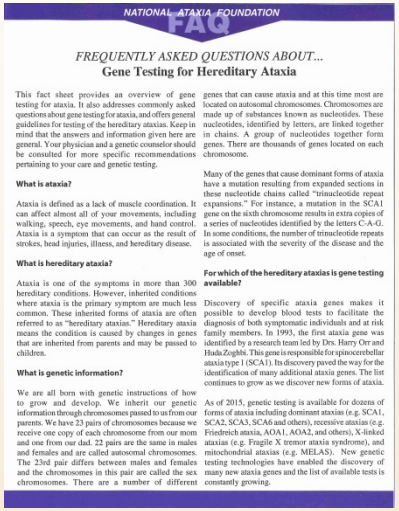
Cerebellar ataxia is classified by its cause (see Figure 1). Unfortunately there are many causes and the terminology can become confusing. In general, there are two main classes of ataxia, acquired and genetic. Two additional classes of ataxia, idiopathic and unknown, are also sometimes used. It is not uncommon for patients to undergo detailed medical evaluations before their cause is known and their ataxia can be properly classified.

Figure 1

Acquired ataxia has an external cause, meaning a person develops it because of something that happens during their life. This can include many different problems and events such as vitamin deficiencies, autoimmune conditions, some infections, exposures to toxic substances or drugs (especially alcohol), various cancers, and many many more. Acquired ataxias often appear "out of nowhere" and the medical term used for this is **sporadic**. Unfortunately, ataxias from all other categories can also occur sporadically, so this term is not useful to categorize the cause of the ataxia. Acquired ataxias are important to recognize early because, in some cases, they may be treatable.

Genetic ataxia has an internal cause, meaning it is due to inherent damage in a person's DNA (their "genetic blueprint"). A person's DNA is made up of 23 pairs of chromosomes (one pair each from their mother and their father) containing sets of genes which together code for all the information which makes a person who they are. People each have over 20,000 genes and we now know of many specific examples where damage to a certain gene causes cerebellar ataxia to develop. Genetic ataxias often run in families (some doctors may say it is a **hereditary** or **familial** ataxia when this occurs) but can also be sporadic without a known family history.

- If an ataxia passes from generation to generation it is said to be **dominant** and is due to a single copy of a defective gene passing from parent to child. Dominant ataxias are most often seen in adults and some can get worse each successive generation. The most well-known dominant ataxias are the spinocerebellar ataxias (or SCAs) such as SCA1, SCA2, SCA3, SCA6, and SCA7, which are each caused by defects in specific genes. SCA3 is the most



NATIONAL ATAXIA FOUNDATION

FAQ

FREQUENTLY ASKED QUESTIONS ABOUT...

Gene Testing for Hereditary Ataxia

This fact sheet provides an overview of gene testing for ataxia. It also addresses commonly asked questions about gene testing for ataxia, and offers general guidelines for testing of the hereditary ataxias. Keep in mind that the answers and information given here are general. Your physician and a genetic counselor should be consulted for more specific recommendations pertaining to your care and genetic testing.

What is ataxia?

Ataxia is defined as a lack of muscle coordination. It can affect almost all of your movements, including walking, speech, eye movements, and hand control. Ataxia is a symptom that can occur as the result of strokes, head injuries, illness, and hereditary disease.

What is hereditary ataxia?

Ataxia is one of the symptoms in more than 300 hereditary conditions. However, inherited conditions where ataxia is the primary symptom are much less common. These inherited forms of ataxia are often referred to as "hereditary ataxias." Hereditary ataxia means the condition is caused by changes in genes that are inherited from parents and may be passed to children.

What is genetic information?

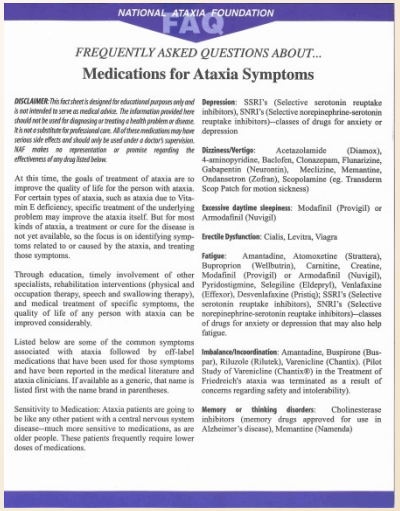
We are all born with genetic instructions of how to grow and develop. We inherit our genetic information through chromosomes passed to us from our parents. We have 23 pairs of chromosomes because we receive one copy of each chromosome from our mom and one from our dad. 22 pairs are the same in males and females and are called autosomal chromosomes. The 23rd pair differs between males and females and the chromosomes in this pair are called the sex chromosomes. There are a number of different genes that can cause ataxia and at this time most are located on autosomal chromosomes. Chromosomes are made up of substances known as nucleotides. These nucleotides, identified by letters, are linked together in chains. A group of nucleotides together form genes. There are thousands of genes located on each chromosome.

Many of the genes that cause dominant forms of ataxia have a mutation resulting from expanded sections in these nucleotide chains called "trinucleotide repeat expansions." For instance, a mutation in the SCA1 gene on the sixth chromosome results in extra copies of a series of nucleotides identified by the letters C-A-G. In some conditions, the number of trinucleotide repeats is associated with the severity of the disease and the age of onset.

For which of the hereditary ataxias is gene testing available?

Discovery of specific ataxia genes makes it possible to develop blood tests to facilitate the diagnosis of both symptomatic individuals and at risk family members. In 1993, the first ataxia gene was identified by a research team led by Drs. Harry Orr and Huda Zoghbi. This gene is responsible for spinocerebellar ataxia type 1 (SCA1). Its discovery paved the way for the identification of many additional ataxia genes. The list continues to grow as we discover new forms of ataxia.

As of 2015, genetic testing is available for dozens of forms of ataxia including dominant ataxias (e.g. SCA1, SCA2, SCA3, SCA6 and others), recessive ataxias (e.g. Friedreich ataxia, AOA1, AOA2, and others), X-linked ataxias (e.g. Fragile X tremor ataxia syndrome), and mitochondrial ataxias (e.g. MELAS). New genetic testing technologies have enabled the discovery of many new ataxia genes and the list of available tests is constantly growing.



NATIONAL ATAXIA FOUNDATION

FAQ

FREQUENTLY ASKED QUESTIONS ABOUT...

Medications for Ataxia Symptoms

DISCLAIMER: *This fact sheet is designed for educational purposes only and is not intended to serve as medical advice. The information provided here should not be used for diagnosing or treating a health problem or disease. It is not a substitute for professional care. All of these medications may have serious side effects and should only be used under a doctor's supervision. NAF makes no representation or promise regarding the effectiveness of any drug listed below.*

At this time, the goals of treatment of ataxia are to improve the quality of life for the person with ataxia. For certain types of ataxia, such as ataxia due to Vitamin E deficiency, specific treatment of the underlying problem may improve the ataxia itself. But for most kinds of ataxia, a treatment or cure for the disease is not yet available, so the focus is on identifying symptoms related to or caused by the ataxia, and treating those symptoms.

Through education, timely involvement of other specialists, rehabilitation interventions (physical and occupation therapy, speech and swallowing therapy), and medical treatment of specific symptoms, the quality of life of any person with ataxia can be improved considerably.

Listed below are some of the common symptoms associated with ataxia followed by off-label medications that have been used for those symptoms and have been reported in the medical literature and ataxia clinicians. If available as a generic, that name is listed first with the name brand in parentheses.

Sensitivity to Medication: Ataxia patients are going to be like any other patient with a central nervous system disease—much more sensitive to medications, as are older people. These patients frequently require lower doses of medications.

Depression: SSRI's (Selective serotonin reuptake inhibitors), SNRI's (Selective norepinephrine-serotonin reuptake inhibitors)--classes of drugs for anxiety or depression

Dizziness/Vertigo: Acetazolamide (Dianox), 4-aminopyridine, Baclofen, Clonazepam, Flunarizine, Gabapentin (Neurontin), Meclizine, Memantine, Ondansetron (Zofran), Scopolamine (eg. Transderm Scop Patch for motion sickness)

Excessive daytime sleepiness: Modafinil (Provigil) or Armodafinil (Nuvigil)

Erectile Dysfunction: Cialis, Levitra, Viagra

Fatigue: Amantadine, Atomoxetine (Strattera), Buproprion (Wellbutrin), Carnitine, Creatine, Modafinil (Provigil) or Armodafinil (Nuvigil), Pyridostigmine, Selegiline (Eldepryl), Venlafaxine (Effexor), Desvenlafaxine (Pristiq); SSRI's (Selective serotonin reuptake inhibitors), SNRI's (Selective norepinephrine-serotonin reuptake inhibitors)--classes of drugs for anxiety or depression that may also help fatigue.

Imbalance/Incoordination: Amantadine, Buspirone (Buspar), Riluzole (Rilutek), Varenicline (Chantix). (Pilot Study of Varenicline (Chantix®) in the Treatment of Friedreich's ataxia was terminated as a result of concerns regarding safety and intolerability).

Memory or thinking disorders: Cholinesterase inhibitors (memory drugs approved for use in Alzheimer's disease), Memantine (Namenda)

National Ataxia Foundation

NAF exhibited, supported, and participated in various meetings:

- **Pentagon CFC Charity Fair held at the Pentagon:** Arlington, VA – January 8
- **American Society for Experimental NeuroTherapeutics (ASENT):** Bethesda, MD – February 20-22
- **Sanford Rare Disease Day Symposium:** Sioux Falls, SD - February 28
- **Rare Disease Day Community Event:** Jacksonville, FL- February 28
- **Abilities Expo:** Los Angeles, CA – February 28 – March 2
- **Abilities Expo:** Atlanta, GA– March 14-16
- **Brain Health Fair:** Philadelphia, PA– April 26

National Ataxia Foundation

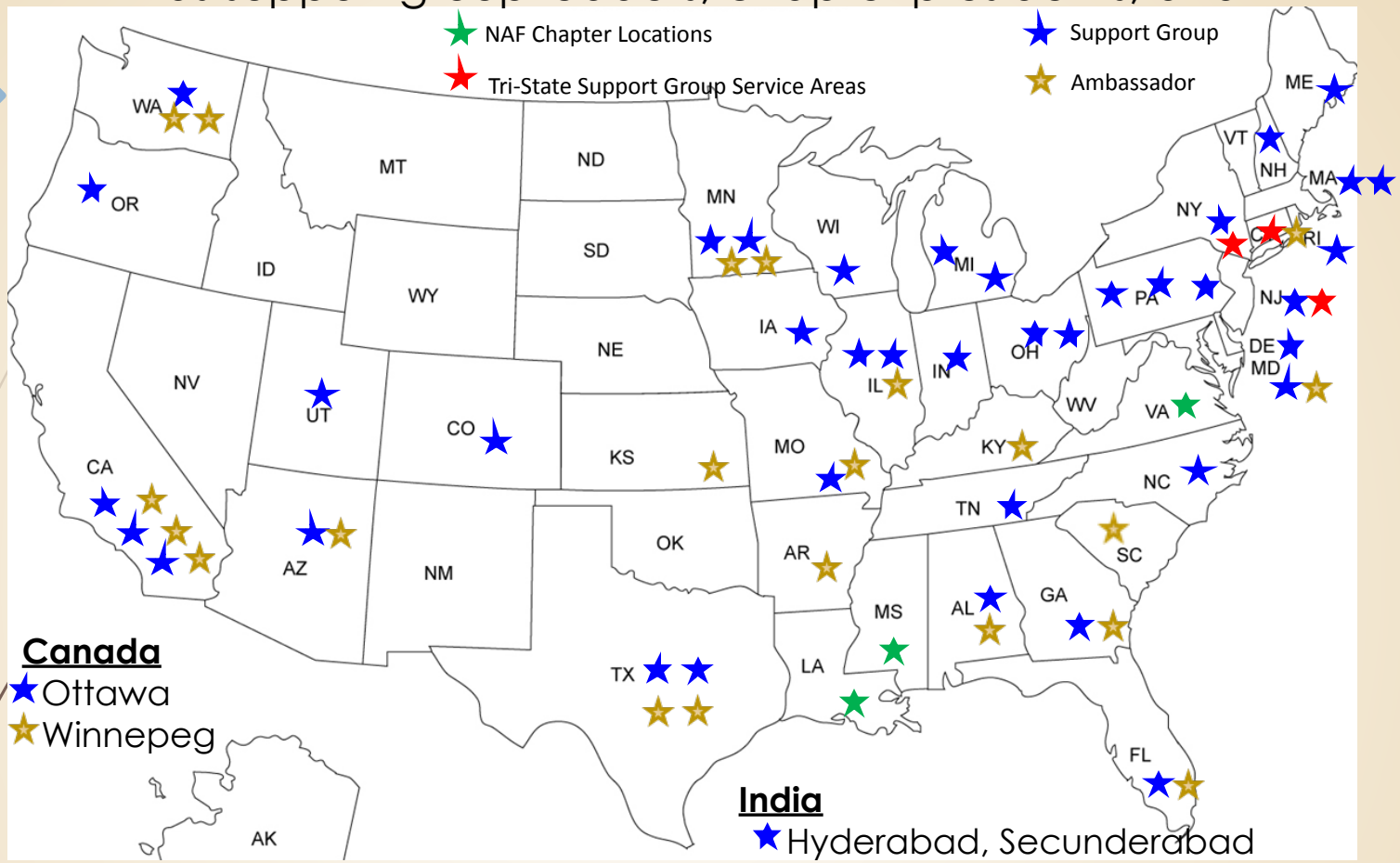
- **American Academy of Neurology Meeting:** Philadelphia, PA– April 28 – May 1
- **Pennsylvania Galleria Community Day Fundraiser and Awareness Event:** Johnston, PA– May 20
- **North Syracuse Family Festival Fundraiser and Awareness Event:** North Syracuse, NY – May 24
- **Metlife Center for Special Needs Planning Financial Planner Symposium:** San Diego, CA – July 29
- **Eastern Panhandle CFC Kickoff Event:** Martinsburg, WV – August 27
- **Abilities Expo:** Boston, MA– September 5-7
- **Central Virginia Area CFC Agency Fair Event:** Richmond, VA – September 23
- **NINDS Non-Profit Forum:** Bethesda, MD– September 23-24
- **North Alabama Nurse Practitioners Annual Symposium:** Huntsville, AL– September 26

National Ataxia Foundation

- **John Hopkins University Ataxia Ambassadors Arts for Ataxia Picnic Awareness Event:**
  Baltimore, MD– September 28
- **King County CFC Kickoff Charity Fair Event:**
  Seattle, WA– October 8
- **Thomas Jefferson Area CFC Charity Fair Event:**
  Charlottesville, VA– November 6
- **John Hopkins University Movement Disorders Symposium:**
  Lutherville-Timonium, MD– November 8
- **HB Cares Charity Event:** Bloomington, MN – November 20
- **Abilities Expo:** San Jose, CA – November 21-23

National Ataxia Foundation

The NAF has a large network of amazing volunteers who serve as support group leaders, chapter presidents, and



ambassadors. These volunteers help identify important local resources and professional care for people with ataxia and their families. Leaders also help coordinate ataxia awareness activities and events.

In 2014, the Foundation had 3 chapters, 41 support groups, and 23 ambassadors providing information, networking and support to local ataxia families.

Walk n' Rolls

- Thank you to all the organizers, donors, participants, sponsors, and volunteers for making these events so successful!

To support promising research and to provide important programs and services takes a national community of amazing volunteers and donors, a dedicated board and staff, a committed medical and research advisory board, and devoted support group leaders, chapter presidents, and ambassadors. We are also thankful to all of our partners, sponsors, corporate and foundation friends, and to all who participate and organize awareness events.

You are all agents of change…

You are all making a difference…

Together we can change the world!!

Thank You!

National Ataxia Foundation