

FARA FACTS

About FARA

The Friedrich's Ataxia Research Alliance (FARA) is a national, public, 501(c)(3), non-profit, tax-exempt organization dedicated to curing Friedrich's ataxia (FA) through research. FARA grants and activities provide support for basic and translational FA research, pharmaceutical/ biotech drug development, clinical trials, and scientific conferences. FARA also serves as a catalyst, between the public and scientific community, to create worldwide exchanges of information that drive medical advances.

FARA was founded in September 1998 by a group of patient families and three of the world's leading FA scientists — Drs. Rob Wilson, Bronya Keats, and Massimo Pandolfo.

Founded: 1998

Country: USA

Status: 501(c)(3) non-profit organization

Focus: A research alliance that drives treatments and a cure for Friedrich's Ataxia

Tax ID #: 52-2122720

www.cureFA.org

Locations:

FARA Headquarters

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Chief Scientific Officer

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Chief Scientific Officer

Raychel Furr Bartek

Co-Founder and Patient- Family Liaison

Felicia DeRosa, MPA

Program Director

Kyle Bryant, Founder, Ride Ataxia

Ride Ataxia Director, FARA Spokesperson

Jamie Young

Office Manager

FARA FACTS *continued*

Mission

FARA's Mission is to marshal and focus the resources and relationships needed to cure FA by raising funds for research, promoting public awareness, and aligning scientists, patients, clinicians, government agencies, pharmaceutical companies and other organizations dedicated to curing FA and related diseases.

Strategy

FARA focuses on grant making for FA research and building collaborations with organizations dedicated to advancing treatments for FA. Due to the progressive nature of the disease and the promise of treatments in development, there is real urgency to our efforts. Directing attention and resources to FA research and partnering with others that share this commitment, FARA believes it can help bring forward effective treatments and a cure for FA.

FARA's Approach to Treatment

Thanks to the committed efforts of many FA scientists, we now understand the cause of FA and specific mechanisms leading to damage in patients, such as the gene mutation, decreased frataxin production, iron sulfur cluster formation, and mitochondrial dysfunction. FARA is supporting the development of treatments aimed at each of these different mechanisms of damage. Because it is based on solid basic science discovery, this targeted approach to treatment has great potential. Furthermore, FA researchers believe that treatment will come in the form of a “cocktail” therapy - meaning that therapies aimed at the different mechanisms of damage have the potential to be used in conjunction with one another to treat the disorder.



Above, Dr. Grazia Isaya and Zac Zies at The Mayo Clinic, Rochester. FARA has funded Dr. Isaya's seminal work on the function of frataxin, the protein that is deficient in FA patients. Dr. Isaya also inspired FARA to begin work on newborn screening for FA. Future newborn screening will allow patients to be treated before the symptoms of FA appear.

FARA FACTS *continued*

FARA's Activities

- Raise funds for advancing research in FA.
- Develop awareness for FA within the general public, scientific and medical professions and the pharmaceutical industry.
- Facilitate a competitive and highly regarded 24/7/365 grant making program that supports greater than 2 million dollars in research, annually.
- Promote the collaborative exchange of information within the scientific community through conferences and networks.
- Manage an FA patient registry that provides researchers with critical patient data and expedites patient recruitment for clinical trials.
- Support the development of a collaborative clinical network that facilitates clinical trials and provides patients with the highest level of clinical care.
- Foster public-private partnerships with the active participation of academic research investigators, government agencies, pharmaceutical companies, advocacy organizations, patients and patient families.
- Rally patients, patient families, scientific investigators, healthcare providers, pharmaceutical companies, government entities and other non-profit organizations to be supporters and advocates for scientific advancements that will lead to treatments and a cure.



Above, Kyle Bryant and several members of the Ride Ataxia III team that rode 191 miles in four days from Portland, Oregon to Seattle, Washington. Ride Ataxia has become the marquee fundraising event for FARA as of 2010.

FARA Leadership

2012 Board of Directors

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Retired U.S. Government Official; Business Consultant

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Founder and Chief Executive Officer, CorVirtus

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Surgeon, Children's Hospital of Philadelphia

Nicholas A. Johnson

Associate & Senior Mechanical Engineer,
Bard, Rao + Athanas Consulting Engineers, LLC, retired

Laura Kalick

Nonprofit Tax Director, BDO Seidman, LLP

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Senior Vice President, USF Health

Paul Marcotte

Attorney & Communications Consultant

Peter Pitts

Partner and Director, Global Healthcare, Porter Novelli
President and Co-Founder, Center for Medicine in the Public Interest

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Medical Director, Translational Neurology, Biogen Idec, Inc

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Chief Scientific Officer, FARA

Professor, Weill Cornell Medical College

Bronya Keats, PhD

Chief Scientific Officer, FARA

Professor, Australian National University

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Johnson & Johnson

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Professor of Biochemistry/Molecular Biology
and Pediatrics, Mayo Clinic Rochester

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Albany Medical College

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of Pennsylvania

Dr. Mark Payne

Professor of Pediatrics (Cardiology)
Indiana University School of Medicine

Dr. Bernard Ravina

Associate Professor of Neurology
University of Rochester

Dr. Giovanna Spinella

Science and Program Consultant,
Former Director of External Research
NIH-Office of Rare Disease
Research

Dr. Robert Wilson, PhD

Associate Professor of Pathology and Laboratory
Medicine, University of Pennsylvania

Making Progress Urgent

Since its founding in 1998, because of the focused efforts and dedication of so many individuals, families, organizations and institutions, FARA has accomplished far more than the research community expected:

- ❶ Assembled and grew the FA scientific community
 - First-of-kind International Scientific Conferences on FA.
 - 1999 (80 scientists, 1 other advocacy group, 0 drug companies)
 - 2003 (100 scientists, 4 other advocacy groups, 4 drug companies)
 - 2006 (150 scientists of 180 applicants, 6 advocacy groups, 6 drug companies)
 - 2011 (200 scientists, 10 advocacy groups, 7 drug companies)
- ❷ Organized issue focused summit meetings engaging additional scientific disciplines and disease groups.
 - Biomarker Summit 2006, Cardiac Summit 2007, Mitochondrial Summit 2008, Therapeutics Symposium 2009
 - Tripled the number of scientists working on FA.
- ❸ Supported basic, translational and clinical research with increasing emphasis on moving to clinic.
 - Grant proposals received per year grew from 1-2 to more than 40
 - Cumulative Grant funding reached over \$14 Million in 2010
 - Total funding facilitated is over \$25 Million
- ❹ Established essential clinical research infrastructure to support clinical trials.
 - Patient Registry
 - Collaborative Clinical Research Network in FA
 - Natural history studies
 - Cell and DNA repositories
 - Clinical outcome and performance measures
- ❺ Established, nurtured and grew powerful public-private partnerships, becoming a recognized model in the field of collaborative research.



Advancing FA Clinical Trials

- 2001 Phase I Idebenone- Complete
- 2005 Phase II Idebenone- Complete
- 2006 Phase II Pilot trial EPO—Complete
- 2008 Phase III Idebenone, US—Complete
- 2008 Phase I A0001— Complete
- 2008 Phase III Idebenone, Europe— Complete
- 2009 Phase II Deferiprone — Complete
- 2009 Phase II Varenicline – Complete
- 2009 Phase II Pioglitazone—Underway in France
- 2009 Phase II Pilot cEPO—Complete
- 2010 Phase II A0001—Complete
- 2011 Pilot study of Resveratrol— Enrolling
- 2011 Phase I, HDACi and Phase IIb/III A0001—Anticipated
- Additional drug candidates are being supported by FARA through direct funding and collaborative support

Funding the Cure

Thank you for your interest in the Friedreich's Ataxia Research Alliance and our quest for the cure. After nearly 12 years, FARA has propelled Friedreich's Ataxia research from a small handful of researchers worldwide and little understanding of the disease to a worldwide movement that is systematically defining the disease mechanisms and creating targeted opportunities to address them.

The number of grants issued by FARA has nearly doubled within the last three years. Within the last six years, the number of clinical trials in FA has gone from one (1) to eight, with more expected before 2010 is through.

The pace of research is more urgent than ever and the cost of research continues to grow as we move closer to treatments and a cure. With a limited patient population, FARA needs support beyond its families and their friends and colleagues. We need your help.

Your gift of support can help spur worldwide collaboration on FA, fund more research, expand the number of patients in clinical trials, encourage more talented scientists to focus on FA and help move us closer to the cure.

General donations support all aspects of the FARA program including:

- Ongoing Basic Scientific Research
- Drug Development
- Clinical Research
- Awareness, Education and Outreach
- Expanded Scientific Expertise
- Fundraising and Development
- Patient Registry
- Scientific Partnerships
- New Research Technologies
- Professional Staff and Program Development

*“Acting alone,
there is very little we can
accomplish.*

*Acting together,
there is very little we will NOT
accomplish!”*

Ron Bartek, FARA Founder and President

Funding the Cure — Summary of Giving Opportunities

Thank you for your interest in the Friedreich’s Ataxia Research Alliance and our quest for the cure. The giving opportunities below will help you identify the type and range of gift that you may be interested in. Additional information describing each giving opportunity follows in this package.

If you would like more information on a particular area or are interested in supporting a part of our program that you do not see, please contact us at (484) 875-3015.

FARA Giving Opportunity Summary	
Fund total per-patient costs for one patient during a clinical trial	\$12,000
Annual cost to maintain and grow the FARA FA patient registry	\$15,000
Funding the 2010 Cardiac Summit	\$15,000
Matching the Keith Michael Andrus Memorial Fund (Cardiac Research)	\$35,000
Annual Funding needed for The Keats Award for International Collaboration	\$50,000
Fund six months of breakthrough development of FA nerve & cardiac cells	\$50,000
Super-Matching (2:1) the Keith Michael Andrus Memorial Fund (Cardiac Research)	\$70,000
Fund FARA’s Annual Education, Awareness and Outreach Program	\$100,000
Fund the FARA-Jackson Lab Partnership (development of mouse models)	\$125,000
Annual Funding needed for New Investigator Awards (2) (\$100,000/year for two years) - encouraging new researchers to join our cause	\$200,000
Fund a year of FARA’s overall program in developing cell models	\$250,000
Sponsor the 2011 International Conference	\$300,000
Fund a 6-month clinical trial	\$500,000
Fund a 12-month clinical trial	\$1,000,000
Fund a 2-year clinical trial	\$2,000,000

Funding the Cure —Your Guide to Giving

This information outlines your options for donating to FARA. If you do not see an option that you would like to review with us, please contact Jennifer Farmer, Executive Director, at (484) 879-6160. We are delighted to consider naming any giving opportunity that is fully funded by an individual or family.

How to Donate

FARA accepts personal donations by phone, fax, mail or online. If you would like to make an online donation, please go to:

www.curefa.org/donate.html

Checks can be mailed to:

Friedreich's Ataxia Research Alliance (FARA)
533 W. Uwchlan Ave
Downingtown, PA 19335
Phone: (484) 879-6160

Monthly Giving

When you make an on-line donation through FARA's website, you have the option to designate a one time, monthly or quarterly gift (found in the gift information section of the transaction form). Your transaction is safe, secure, and private. The online credit card processor charges 3.3% of your donation as a processing fee.

Stock Donations

FARA welcomes wired and stock donations. Bank fees are typically \$15 - \$25 per transaction. You can find this information at www.curefa.org or contact Jennifer Farmer at (484) 879-6160

Special Occasion Donations

Turn your next special occasion into a valuable way to support FARA's important work towards finding treatments and a cure for Friedreich's ataxia. Gifts can be made through online donations, mailing checks or via credit card. Consider a special occasion gift whenever you are planning:

- Birthdays
- Anniversaries
- Weddings
- Holiday Parties
- Retirements

Memorials

We are honored that you would consider a donation to FARA in memory of a loved one. Memorial Donations can be made in all of the methods described above. For checks, please designate the name of the individual in your check or correspondence and add ATTN: Memorials on the outside of your envelope.

FARA Donor Bill of Rights

PHILANTHROPY is based on voluntary action for the common good. It is a tradition of giving and sharing that is primary to the quality of life. To ensure that philanthropy merits the respect and trust of the general public, and that donors and prospective donors can have full confidence in the Friedreich's Ataxia Research Alliance (FARA), FARA declares that all donors have these rights:

- I. To be informed of FARA's mission, of the way FARA intends to use donated resources, and of its capacity to use donations effectively for their intended purposes.
- II. To be informed of the identity of those serving on FARA's governing board and staff leadership, and to expect the board and leadership to exercise prudent judgment in their stewardship responsibilities
- III. To have access to FARA's most recent financial statements.
- IV. To be assured their gifts will be used for the purposes for which they were given.
- V. To receive appropriate acknowledgement and recognition.
- VI. To be assured that information about their donations is handled with respect and with confidentiality to the extent provided by law.
- VII. To expect that all relationships with individuals representing FARA will be professional in nature.
- VIII. To be informed whether those seeking donations are volunteers or employees of FARA. FARA does not hire outside paid solicitors.
- IX. To have the opportunity for their names to be deleted from mailing lists
- X. To feel free to ask questions when making a donation and to receive prompt, truthful and forthright answers.

Fund Clinical Trials — Shots on Goal

To achieve our goal to slow, stop, reverse and cure FA, FARA must successfully navigate the financial and logistical obstacles presented by clinical trials. The Food and Drug Administration and other regulatory agencies around the world use the data from clinical trials to determine if a drug works in a particular disease and if the benefits shown in the trial outweigh the risks demonstrated in the trial. A number of promising FA clinical trials are already under way and FARA is deeply involved in preparing the launch of additional trials. FARA will continue filling its discovery pipeline and supporting new clinical trials until we achieve treatments and a cure.

Clinical trials are very expensive. The cost of the drugs must be covered; the clinical trial investigators and staff must be paid; payment must be made for the materials and personnel needed to conduct all the tests required and to analyze the test results, and the participating patients must be transported to the clinical sites, lodged and fed. That final requirement is especially important because most clinical trials fail and most fail because of insufficient patient participation.

We cannot allow such failures to occur in FA despite the challenges of recruiting sufficient patient participants in a rare disease. We must take full advantage of every shot on goal – every clinical trial – we can muster.

FARA attempts to match every promising drug discovery with a pharmaceutical partner willing to share the costs of drug development and clinical trials. FARA also continues to seek other advocacy organizations as partners in supporting clinical trials. FARA must be prepared to do its part in these partnerships and to bear the bulk of the costs when partnerships prove impossible.

Help FARA Advance our most promising treatments into clinical trials. FARA is planning to support the launch of five additional clinical trials in 2010. These trials include our most promising prospects for treatments to date. We need your help to ensure that FARA is able to provide the support needed to conduct these vital clinical trials.

You can help:

Fund transportation, lodging and meals for one patient in a clinical trial

..... **\$2,500**

Fund total per-patient costs for one patient during a clinical trial \$12,000

Fund a 6-month clinical trial
..... **\$500,000**

Fund a 12-month clinical trial
..... **\$1,000,000**

Fund a 2-year clinical trial
..... **\$2,000,000**

FARA's Patient Registry—An Invaluable Research Tool

FARA's patient registry is the critical link to successful research and is invaluable in enabling and accelerating FARA's remarkably active clinical trial programs. Here's why:

Most clinical trials fail. In most cases, failure results from insufficient patient participation. Many disease groups have no patient registry so have great difficulty identifying and contacting patients to inform them of trials. Drug companies often spend millions of dollars recruiting patients for a clinical trial. They also spend a lot of time doing so – it is not unusual for companies to devote two years to recruitment. Even after all that time and money is spent, recruitment is often unsuccessful.

In Friedreich's ataxia, we cannot allow that to happen to us. FARA is urgently pursuing treatments and a cure and cannot spend years recruiting patients for a clinical trial. Nor can we permit FA clinical trials to fail because of insufficient patient participation. Especially because FA is a rare disease, if we permitted a clinical trial to fail because not enough patients participated, the sponsoring drug company would certainly not come back to us to try that drug or any other drug in its inventory in FA.

Enter the FARA patient registry. With it, we have been able to recruit sufficient FA patients for clinical trials in weeks, not months or years, and none of the FA trials have failed for lack of sufficient participants. And, the registry has had a very positive multiplier effect in two ways. First, because FARA's patient recruitment saves the drug companies a lot of time and money, more and more drug companies are interested in working with FARA on FA. Second, because FARA's patient recruitment is so effective, drug companies are able to conduct their trials with more FA patients, increasing the effectiveness of the trials significantly.

FARA is also using its patient registry very effectively in recruiting patients willing to participate in research important in establishing the basis for clinical trial. These patients are providing various biological samples, participating in clinical examinations, and helping establish the natural history of FA and the baseline for clinical drug trials.

FARA's patient registry is the largest FA registry in the world and is the only worldwide FA registry. It is considered a model for other rare disease advocacy registries and FARA representatives serve on the NIH panel spearheading development of a global rare disease patient registry.

FARA's patient registry has come to represent a first ray of hope for newly diagnosed FA patients and their families. As a result of FARA's diligent effort with the primary FA diagnostics lab, when FA patients in the United States are first diagnosed and given the news that they have a devastating progressive disease for which there are no treatments or cure, they are also provided hope -- a letter telling them about FARA and the patient registry and introducing them to the opportunity to participate in the quest for a cure through the patient registry and clinical research.

We need your help to propel FA research further and faster by supporting the maintenance and growth of this vital element of our research program.

Annual cost to maintain and grow the FARA FA patient registry..... \$15,000

"We here at Santhera know very well that our ambitious target of 70 patients into the US Phase III study with idebenone (IONIA trial) would have never been accomplished without the active and continuous support by FARA's Patient Registry and FAPG."

Thomas Meier, PhD
Chief Scientific Officer, Santhera

Select the Most Promising Drug Candidates

One of the most important steps in developing new drugs for FA treatments is determining which formulation or configuration of the candidate drug is most likely to work best. The tools for making this determination are the FA cells, assays, cell models and animal models used to “screen” such drugs. Such screening usually begins in human cells that originate with FA patients and their families. It can also begin with the use of cells (called assays) from organisms such as yeast or animals into which some of the features of FA have been introduced.

To provide FA investigators and pharmaceutical companies the human cells they need for drug screening, FARA provides “immortalized” cell lines, maintained in an international repository, available to all FA investigations. FARA also continues to organize such blood draws to provide fresh blood cells for drug screening.

Breakthrough in developing FA nerve and muscle cell models --

Taking advantage of exciting new breakthroughs in medical technology, FARA is now supporting the development of human cell models that are to include nerve and heart muscle cells. This is extremely important for drug screening because the human cells currently available for drug screening are primarily blood cells rather than the cells in which FA primarily is manifested – nerve and muscle cells.

Important partnerships in developing better FA mouse models –

As is the case in many diseases, FA investigators have worked for years, with FARA support, to develop animal models of FA in which new drug discoveries can be tested and the very best drug candidates can be selected for advancement into human FA clinical trials. As a result, we have mouse models that involve some of the genetic and biological characteristics of FA and have been somewhat helpful in screening and evaluating drugs. However, these mouse models are far from perfect and must be improved so we can make far faster progress in advancing drugs into human clinical trials. To accomplish this important task, FARA and FA investigators have teamed with a world-class partner – The Jackson Laboratory in Bar Harbor, Maine. Our colleagues there are importing our existing mouse models to their facility (the world’s largest animal-model facility) and will work on improving these models significantly and distributing them to the FA investigators and pharmaceutical companies that need them to screen drugs and identify the best drug candidates for human clinical trials.

With your help, FARA can test more drugs, more quickly and with greater accuracy:

Fund six months of breakthrough development of FA nerve & cardiac cells
.....\$50,000

Fund the FARA-Jackson Lab Partnership
.....\$125,000

Fund a year of FARA’s overall program in developing cell models.....
.....\$250,000

Give a Heart Eliminate Life-Shortening Heart Disease in FA

Most FA patients have heart problems. For many of them, their heart condition is the most serious and life-threatening FA symptom. Yet, the heart condition caused by FA is not well understood and, to date, has not been sufficiently investigated. Scientists have not identified a clear path to therapy specific to FA hearts. Consequently, cardiologists attempt to treat the symptoms of FA hearts with the same medications used to treat heart disease in the general population. The result is that we are losing far too many FA patients to heart failure. In most FA patients, even when the heart is not the direct cause of death, the heart condition contributes significantly to the diminished level of health and to shortened life.

We need your help in supporting FA heart research along two vital tracks simultaneously:

- ❶ FA scientists are eager to determine which of the current heart treatments are best suited for addressing the symptoms of FA hearts.
- ❷ Scientists are also eager to accelerate progress in developing a better understanding of the underlying causes of FA heart disease.

FARA has established a world-class cardiac panel consisting of the heart specialists that know FA best. This panel meets again in the summer of 2010 to decide how best to accelerate progress on both FA cardiac tracks. At that meeting, FARA will announce its intent of awarding at least two major grants this year to advance FA cardiac research – one grant on each of the two tracks.

We are losing young FA patients to heart failure. We must figure out how best to treat their heart symptoms right away and how to accelerate progress toward curing their heart disease as soon as possible.

“The American Heart Association has helped fund studies on heart function in animal models of FA and in patients. This is a significant recognition by a large and definitive scientific group that the heart is a key organ affected in FA and needs greater study. FARA’s heightened emphasis on research into heart function in FA patients will help determine three things:

- 1) The mechanism for heart dysfunction in this disease.
- 2) How we can improve heart function in this disease to improve lifespan and decrease mortality
- 3) Why some hearts are affected and others are not. This may offer clues about gene expression that can be taken advantage of to develop new therapies.”

*Dr. Mark Payne, Cardiologist, FA Researcher,
Indiana University School of Medicine*

On January 22, 2010, the son of FARA co-founders Ron and Raychel Bartek, Keith Michael Andrus, who had been diagnosed with FA at age 11, died of congestive heart failure one month after his 24th birthday. FARA decided immediately to ask that any donations made in Keith’s memory be placed in a fund to be used to support FA heart research.

The Keith Michael Andrus Memorial Fund has grown to \$35,000. We need your help to grow that fund so FARA can award the two grants needed to advance this critical heart research along both important tracks. Please consider:

Matching the Keith Michael Andrus Memorial Fund \$35,000

Super-Matching (2:1) the Keith Michael Andrus Memorial Fund \$70,000

Foster International Scientific Collaboration

Nothing is more powerful in propelling FA research forward at increasing rates than collaboration. Because FARA has always supported promising FA science around the globe, the sun never sets on our research and international collaboration is a vital component of the FARA effort.

FARA owes much of its zeal for international collaboration to Dr. Bronya J. Keats. Dr. Keats is a founding member of FARA’s Board of Directors and Scientific Review Committee and long served as its scientific director. She also made monumental contributions to establishing FARA, instructing its operations, shaping its research portfolio, educating its lay participants and advancing FA research. She has kept FARA’s compass set on the most meritorious research that will lead most effectively and quickly to treatments and a cure.

Throughout her involvement with FARA, Dr. Keats has been influential in fostering and supporting collaboration among scientists from around the world. In her honor, FARA established the **Bronya J. Keats Award for International Collaboration in Research on Friedreich’s Ataxia.**

This Award is given on an annual basis to investigators performing high-quality, promising Friedreich’s ataxia research that relies on international collaboration among scientists.

With your help FARA can continue to attract and fund leading scientists around the world.

Total

Support New Investigators

From its earliest days, FARA has recognized that one of the most important ways it could increase the momentum behind the scientific exploration of FA was by “growing the field.” Because FA is a rare disease and, initially, had only a small number of scientists devoting themselves to FA, we knew we had to expand that number significantly by attracting additional investigators to our cause.

FARA’s success thus far in this important endeavor can be seen in comparing the numbers of participants in our international FA conferences to date. When we organized the world’s first International Scientific Conference on FA in 1999, we struggled to find scientists who were interested in FA and were willing to spend three days discussing FA with us at the National Institutes of Health (NIH), and 15 of those were NIH investigators. When we held the second such conference, in 2003, 100 scientists from 12 countries participated and, for the third conference, in 2006, we had to turn away 30 scientists to hold the meeting to a manageable 150.

FARA believes in welcoming, mentoring and supporting new scientists in the FA research community so that they have the necessary experience and motivation to be successful in advancing FA science. The New Investigator Awards encourage scientists near the beginning of their careers who demonstrate an interest in FA.

They also attract researchers with significant expertise in other areas. These awards provide the opportunity to test new hypotheses and innovative ideas.

Please help FARA attract talented new investigators to FA—Annual Funding needed for New Investigator Awards

..... **two (2) at \$200,000 (\$100,000 per year for two years)**

Build Worldwide Collaboration— FA Conferences, Meetings and Workshops

Sharing insights, ideas, and expertise among all in the research community is essential for helping scientists and drug companies advance their work and for bringing FA treatments forward faster. Conferences, meetings and workshops that bring together FA researchers and scientists in related diseases and specialty areas are proven to generate new collaborations, hypotheses and discoveries. FARA has, from its inception, sponsored, organized and conducted such conferences, meetings and workshops to foster the full and open collaboration needed to accelerate progress toward treatments and a cure.

- ❶ Full-Scale Conferences— FARA sponsors periodic, comprehensive, international conferences like the ones it organized, co-sponsored, and co-hosted with NIH in 1999, 2003 and 2006. These workshops provide the opportunity for the full scientific community involved in all aspects of research related to FA to come together to share findings and insights and to chart the best course to treatments and a cure.
- ❷ Topic-Specific Summit Meetings and Workshops — FARA also supports summit meetings and workshops that are more focused and in-depth on a specific topic area or arranged for smaller groups of investigators to establish collaborations or to organize a specific project that requires multiple collaborators. In 2008, for example, FARA funded, organized and hosted a Mitochondrial Summit that gathered the world’s leading authorities on the kind of mitochondrial dysfunction that contributes to FA, Parkinson’s, Huntington’s, and Alzheimer’s diseases, ALS, diabetes, stroke and many other disorders as well as aging. FARA also held its first Cardiac Summit later that year and, in 2009, held a symposium devoted entirely to the therapeutics in development for FA.

FARA works to minimize costs of these meetings by seeking sponsorships, collaborating with other organizations and institutions and soliciting in-kind donations.

FARA hosts two Summits/Workshops this year. FARA hosted a meeting on cellular research in March of this year and will hold its second Cardiac Summit in June 2010 convening the world’s top cardiac researchers in FA. FARA will host its fourth International scientific conference on FA in spring 2011 and anticipates that 200 leading researchers in FA and other related diseases will participate.

Help foster new collaborations, hypotheses and discoveries by bringing FA’s top researchers together:

**Funding the 2010 Cardiac Summit
..... \$15,000**

Sponsor part or all of the International Conference.....\$300,000

Promote Education, Awareness and Outreach

FARA’s mission includes promoting public awareness and aligning scientists, patients, clinicians, governments agencies, pharmaceutical companies and other organizations dedicated to curing FA and related diseases. Success will come through inspiring and gaining unwavering commitment from all stakeholders, drawing more awareness to our cause and educating all about our program and mission. Here are just a few examples of this outreach:

- 1 Patients—Staying close to our patient community brings them hope, provides critical and timely information on our research progress and educates them on how they can help by participating in scientific studies and clinical trials and fundraising. To accomplish this, FARA staff attends more than 25 grassroots fundraising events each year. We co-host patient symposia, publish a newsletter, provide a web-site and use social media to offer information on our current research progress and living with FA, among many other areas. Through FARA’s patient spokesperson, Kyle Bryant, the ataxia community is inspired to remain active, educated about the benefits of physical activity, and motivated to empower themselves by engaging in activities like recumbent cycling.
- 2 The scientific and research community—The more our current and potential research partners learn, the greater their commitment to become part of the cure. This is true for all professionals involved at every level of our research program. It is not unusual for researchers working in the lab or pharmaceutical company employees and leaders to have little or no direct patient contact. FARA’s outreach initiative brings the human impact of FA to them through opportunities to meet and interact with those who live with FA. To maximize our presence in the research community, FARA attends and presents at professional conferences, addresses research groups, pharmaceutical companies, student and professional groups and others forums.
- 3 Medical Practitioners—Because FA is a rare disease, physicians do not receive training or continuing education on the disease. General neurologists may only encounter one or two patients with FA during their career. FARA’s medical Grand Rounds program brings FA education to the physician. Grand Rounds is a regular teaching symposium held within the hospital for which physicians receive Continuing Medical Education Credits. The Principal Investigators of FARA’s clinical network in FA along with patient spokespersons implement this program around the country.

With the dawn of the treatment era for FA and the growing need to fund an increasing number of clinical trials, it is also imperative to create awareness about Friedreich’s ataxia among the general public.

This year, FARA launched a branded fundraising event called Ride Ataxia. Ride Ataxia was founded by Kyle Bryant as a cycling fundraiser to draw attention to FA and raise funds for research. Now, as part of the FARA team, Kyle and FARA will host Ride Ataxia in multiple locations around the country (4 in 2010).

Ride Ataxia brings together in a family atmosphere all community members of all abilities to draw awareness to the cause, and encourage people of all fitness levels to challenge themselves. Ride Ataxia has already garnered regional and national press and has begun to engage cyclists and supporters from the general public.

Your support will help ensure that patients and families, researchers, medical practitioners and the general public have an opportunity to understand FA and join FARA’s effort to Cure FA. The cost to fund FARA’s Annual Education, Awareness and Outreach is \$100,000

