

# The Advocate

...your connection to the Friedrich's Ataxia Research Alliance



Issue #7

Winter 2007-2008

## Clinical Trials Update

### Friedreich's Ataxia Treatment Pipeline

By Jennifer Farmer

FARA is excited to share with you our confidence and vision for upcoming treatments. We believe that research and drug discovery requires a diverse and strategic approach. Thanks to the brilliant and committed efforts of many FA scientists, we now understand the cause of FA and specific mechanisms leading to the damage in patients. These understandings allow for more targeted approaches to treatment and optimism. Our goal is to foster drug discovery and development so that there are several "shots on goal" that act in different ways and have unique approaches based on known mechanisms.

We anticipate several clinical trials for 2008:

- A phase III study of Idebenone has been approved by the FDA and is expected to begin enrollment in late November/early December at the Children's Hospital of Philadelphia and University of California Los Angeles. The results of the promising phase II study conducted at the NIH were recently published by Nicholas DiProspero, and others, in *Lancet Neurology*. The results of the phase II study revealed that high doses of Idebenone were well tolerated and there was an association with improvement in neurological function. Santhera Pharmaceuticals is sponsoring this trial and FARA is working closely with them to help with patient recruitment through our patient registry.
- A phase I/II study of Deferiprone has been submitted to the FDA and is expected to begin enrollment in early 2008.

*Continued on p. 3*

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## Grassroots Fundraising



*Donovan Simpson and friends at the finish line of the Westchester, NY Triathlon*

By Marilyn Downing

The grassroots fundraising effort continues to grow steadily and it significantly supports many of FARA's research projects. In addition to supporting our scientists, these events also raise awareness of FA in our individual communities.

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## What is Friedrich's ataxia?

Friedreich's ataxia is a life-shortening, debilitating and rare genetic neurodegenerative disorder. Onset of symptoms usually occurs between the ages of 5 and 15. Symptoms include muscle weakness and loss of coordination in the arms and legs; impairment of vision, hearing and speech; aggressive scoliosis (curvature of the spine); diabetes, and a serious heart condition. Most patients need a wheelchair full-time by their late teens or early twenties. There is no cure. Most childhood-onset patients with this disease die in early adulthood. FARA is a 501(c)(3) tax-exempt non-profit organization dedicated to supporting research leading to treatments and a cure for this relentless and devastating disorder.

## President's Message

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)



### Allies in Our Mission

*Dear friends and partners,*

*Never has there been a more powerful assembly of allies working in concert to achieve effective treatment and a cure for Friedreich's ataxia, and additional allies continue to rally to our cause. I am reminded of conversations with FARA co-founder Raychel Furr Bartek as we were selecting a name for the organization in 1998. Raychel believed that "FARA" was the right choice because it would represent a good name – the Friedreich's Ataxia Research Alliance – as well as the organization's primary mode of action – forging a research alliance. With everyone working together, FARA has certainly forged a tremendous, effective alliance that continues to grow in size and momentum.*

*So much so, in fact, that FARA's Board of Directors recently decided to place that central idea into an expanded FARA mission statement. FARA's mission statement had simply been "to slow, stop and reverse Friedreich's ataxia." With the recent reinforcement of the statement, it has been revised to:*

*"FARA's mission is to marshal and focus the resources needed to slow, stop and reverse Friedreich's ataxia by raising funds for research, promoting awareness, and aligning scientists, patients, clinical networks, government agencies, pharmaceutical companies and other organizations dedicated to eradicating Friedreich's ataxia and related neurodegenerative diseases."*

*Many of you are aware of FARA's collaboration with a number of these allies such as the National Institutes of Health, the Muscular Dystrophy Association, the National Ataxia Foundation, and other organizations around the world with whom FARA has agreements to co-fund the most promising FA research. One more invaluable partner has just joined the alliance – the American Heart Association (AHA). Knowing that FA-related cardiology is poorly understood and extremely important to FA patients, FARA con-*

*vened its first "Cardiac Summit" in late August. Dr. R. Mark Payne, a gifted pediatric cardiologist, who has been conducting FA research under a series of FARA and NIH grants, agreed to chair the "Summit" and the FA Cardiology Panel that assembled there for the first time. At FARA's invitation, an AHA representative, Phillip Burks, also participated in the "Summit." FARA-AHA discussions began immediately on collaboration and co-funding of promising FA cardiology research and, in November, a FARA-AHA agreement was signed that will find the two organizations working closely together to solicit and fund the research most likely to lead to a far better understanding of FA-related cardiology and effective treatment of the condition.*

*AHA joins our alliance just as FARA is set to launch the first of at least five FA clinical trials of promising therapeutics over the coming year. Each of these trials will be supported by a pharmaceutical company and will be conducted at centers involved in the Collaborative Clinical Research Network in FA that is co-funded by FARA and MDA. These will depend on adequate participation by FA patients wanting to help move these promising drugs across the finish line of approval. In other words, the single most important ally in this increasingly powerful alliance is the FA patient with the support of the FA patient family. Only with sufficient patient participation can these promising drugs and clinical trials succeed. Your alliance needs you. Please register in FARA's patient registry and be prepared to enroll in clinical trials.*

*On behalf of FARA, I want to thank all of you for your support in 2007. It will help bring us all closer to the finish line in 2008.*

*Warm regards,  
Ron*

## Clinical Trials (continued from page 1)

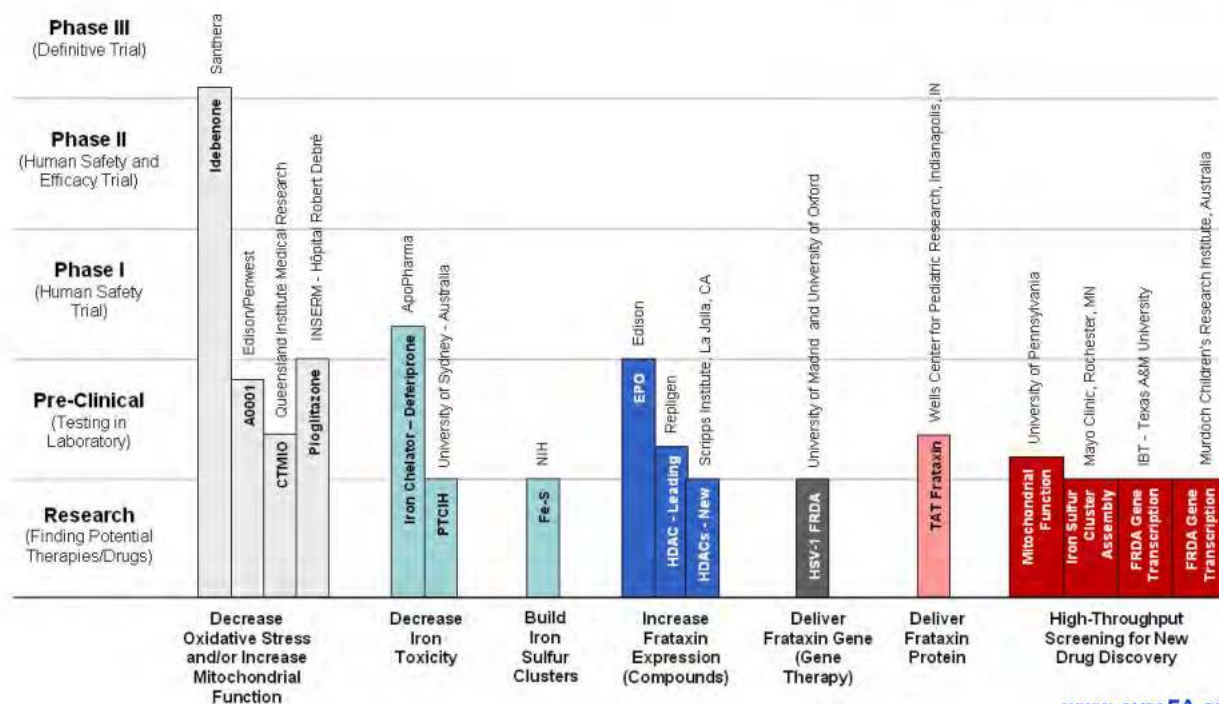
- Deferiprone is an iron chelator that has shown promising results in a pilot study conducted in France. Results were published this year by Nathalie Boddaert in the journal *Blood*. This study will be conducted at multiple sites in the United States and Europe. ApoPharma is sponsoring this trial and FARA is working closely with them to help with patient recruitment through our patient registry.
- Additional trials of A0001 and Erythropoietin (EPO) are also anticipated for 2008. A0001 is a drug discovered by Edison Pharmaceuticals that has its mechanism of action in the mitochondria. Penwest Pharmaceuticals is now advancing this drug to clinical trials. EPO is a hormone produced in our bodies and it is also a drug approved to increase red blood cells. It is commonly used in dialysis and cancer patients and to build up red blood cells prior to surgery. Austrian researchers Drs. Barbara Scheiber-Mojdehkar and Brigitte Sturm found that EPO increases frataxin levels and this past year, completed proof-of-principle studies in patients (in press; accepted by a journal but not yet published). Edison Pharmaceuticals is working with these investigators and the clinical research network to initiate an EPO trial in 2008.
- RepliGen Corporation and Dr. Joel Gottesfeld at Scripps Research Institute continue to test and develop HDAC inhibitors. HDAC inhibitors have their action at the DNA/gene level and increase frataxin in cellular and animal models of FA. Repligen is working to fully evaluate the lead compounds and gather the necessary data for a submission to the FDA (estimated late 2008).
- Throughout the research pipeline are other early stage studies that are designed to investigate new treatments. We believe that we need multiple shots on goal in various areas to ensure successful treatments are delivered to all patients with FA. For example, on the far right of the pipeline are drug discovery studies using a technology called high throughput screening. Each of these studies is different in that the researcher has developed an assay to measure a desired outcome (e.g. improved mitochondrial function, increased frataxin, etc.) and the assay is then used to screen large libraries of compounds to identify "hit compounds" or new drug targets. ■

## Friedreich's Ataxia Pipeline

October 2007

Available to  
Patients

FARA has supported, and is supporting, these efforts by providing various combinations of direct funding, essential clinical infrastructure, advocacy and awareness efforts.





### FARA Funds 17 Grants Exceeds \$1 Million in Research Dollars

By Jennifer Farmer

FARA is excited to share that we approved funding for over \$1 million in research dollars for 17 grants and our partner organizations funded an additional \$500,000 for these meritorious projects.

FARA's competitive research grant program accepts grants on a continuous basis throughout the year from FA scientists around the world. All grant applications go through a scientific peer review process to ensure that we fund the best research.

Over the past year FARA has prioritized funding to support research that directly advances drug targets (translational research) and clinical trials. This type of research requires higher levels of funding. Our ability to fund research is directly dependent on your active support through donations and participation in fundraising.

FARA partners are also committed to advancing FA research and we have been able to partner with them in making several of these grant awards. We would like to recognize our 2007 funding partners:

- Muscular Dystrophy Association
- National Ataxia Foundation
- Friedreich's Ataxia Research Alliance – Australia/New Zealand.

Acting alone there is little we can accomplish but working together there is little we will NOT accomplish.

**See Grants Awarded in 2007 on p. 5**

### FARA Hosts First Cardiac Summit

By Jennifer Farmer

The cardiac complications associated with FA are a major concern to patients and families as they can cause the most severe and devastating outcomes. FARA has identified cardiac research as a priority area. We believe that with increased focus and attention in this area we can achieve better outcomes for patients.

FARA recruited Dr. Mark Payne, pediatric cardiologist and FA researcher from the Indiana University School of Medicine to chair the first meeting of scientists and cardiologists to focus on the impact of FA on the heart. Fourteen cardiologists, researchers and patient advocates, including the American Heart Association (AHA), met for a very productive meeting.

The goals of this meeting were to:

- Establish a working group of scientists and clinicians, chaired by Dr. Payne, focused on heart disease in FA. Use this group to identify and recruit new investigators and leverage database development for new clinical and translational studies.
- Identify and establish a patient database(s) and a registry for multicenter studies / trials exploring FA cardiology.
- Identify and prioritize clinical and basic research goals for the next year regarding cardiac disease in FA.

One major outcome of the Summit was documenting the gaps in knowledge and assigning teams of researchers to begin work in these areas. The Summit team ranked the following cardiac-specific aims in order of their importance and urgency to initiate:

1. Retrospective and prospective evaluation of Mortality and Morbidity in FA patients. Why do FA patients die, and what are the associated complications?
2. Retrospective analysis of cardiovascular biomarkers in FA. This will involve retrieving echocardiograms and EKGs on FA patients for the past 10 years and correlating with patient outcomes. This data is greatly needed to understand the cardiac impact of FA, and generate prognostic markers, such as the importance of T wave abnormalities (EKG changes), and echocardiographic markers of diastolic dysfunction.
3. Initiate longitudinal studies of cardiovascular disease. All patients with the diagnosis of FA need to enter a natural history study that follows specific cardiovascular evaluations.
4. Establish a core cardiac lab for the purpose of setting standards in cardiac measurements across multiple sites and imaging platforms. This lab will be essential to maintain high quality data from different sites.
5. Evaluate how cardiomyopathy impacts the quality of life for the FA patients.
6. Implement basic and translational studies focused on the heart.

Work has begun in multiple areas to further understand cardiovascular disease in FA. To further support these efforts FARA has gained the attention of AHA and the two organizations have entered a partnership agreement to co-fund grants beginning in 2008. An announcement of AHA/FARA Ataxia Cardiomyopathy Research Award will be made in Nov/Dec 2007 and the first grant will be awarded in 2008. Watch 2008 editions of the Advocate for information on the award recipients and progress on cardiac disease in FA. ■

## Grants Awarded in 2007

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)

Principal Investigator	Project	Research Area	Category	Outcome
*Joseph Sarsero	Development of pharmacological therapies for FA using humanized mouse models	Basic / Translational	Animal Model and Drug Screening	↑ frataxin
Pook/Gottesfeld	Histone Deacetylase Inhibitor (HDACI) therapy of a Friedreich's ataxia mouse model	Translational	Drug Development	↑ frataxin
*David Lynch	Collaborative Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
*Grazia Isaya	Modulators of frataxin assembly: assay development for high throughput screening	Translational	Drug Screening	All
Robert Wilson	Supplement to previous FARA grant and RO1 to support high throughput drug screening	Translational	Drug Screening	All
Marek Napierala	Influence of chlorambucil-conjugated GAA-TTC sequence-specific polyamides and Histone Deacetylase Inhibitors (HDACI) on repeat instability and frataxin expression	Basic / Translational	FRDA Gene	↑ frataxin
Filip Lim & Richard Wade-Martins	Neuron - validated approaches for developing Friedreich's ataxia therapeutics	Basic / Translational	FRDA Gene	↑ frataxin
Des Richardson	Iron Chelation Efficacy of Novel PCIH Iron Chelators In Vivo and their Ability to Prevent the Pathology Observed in the Conditional Frataxin Knockout Mouse.	Translational	Iron chelation / Drug Screening	All
*Nuri Gueven	Use of a novel catalytic antioxidant, CTMIO, in a GAA repeat mouse model of FRDA	Translational	Antioxidants	↓ oxidative stress and ↑ mitochondrial function
Arnulf Koeppen	Friedreich's ataxia: Iron dysmetabolism in the central and peripheral nervous systems	Basic	Pathology & Iron Metabolism	Elucidate Pathophysiology
*Martin Delatycki	Supplement to Collaborative Clinical Research Network for Friedreich's Ataxia – Australia site	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
Tracey Rouault and Richard Holms	Development and use of synthetic iron-sulfur clusters in therapy of Friedreich ataxia	Translational	Iron-Sulfur Cluster assembly	↓ oxidative stress and ↑ mitochondrial function
RepliGen Corporation	Preclinical optimization of HDAC inhibitors for treating Friedreich's Ataxia	Translational	FRDA gene	↑ frataxin
Robert Wilson	RNAi therapeutics for Friedreich ataxia	Basic/Translational	FRDA gene	↑ frataxin
*Erika Becker and Des Richardson	Mitochondrial Iron Overload and Friedreich's Ataxia: The Role of Frataxin in Iron Metabolism.	Basic	Frataxin function	Elucidate pathophysiology
Joel Gottesfeld	Improved HDAC inhibitors as Therapeutics for FA	Translational	FRDA gene	↑ frataxin
Bernard Ravina	Supplement Clinical Research Network for Friedreich's Ataxia – Data Coordination Center	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All

\* Grants co-funded with MDA, # grant co-funded with NAF, ^ grant co-funded with FARA-Australia

"Research Area" refers to three broad categories of research -- basic, translational and clinical. Basic research is the most fundamental and explores the underlying causes and mechanisms (pathophysiology) of the disease. Translational (or preclinical) research advances the results of basic research from discovery through development, from "bench to bedside." It involves, for example, testing drug discoveries in animals and human cell cultures in preparation for tests in humans. Clinical research involves trials in humans. The "Category" column is used to characterize the focus of each research project. The "Outcome" column places the goal or long-term benefit of each project into one of four categories – Determine Pathophysiology, Improve Mitochondrial Function (↑), Reduce Oxidative Stress (↓), or Elevate Frataxin Protein levels (↑).

### Note:

In 2008, we will continue to maintain a balanced research portfolio that adds new treatment candidates to our research pipeline and advances the most promising treatments. We want to ensure that leading candidates have the resources for drug development and that we have the essential infrastructure to support clinical trials. ■

## Featured Scientist

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)

### Dr. Bernard Ravina

*Dr. Bernard Ravina is the Director of the Movement and Inherited Neurological Disorders Unit and Associate Director of the Clinical Trials Coordination Center at the University of Rochester. He received his MD degree from Johns Hopkins and has a MS in clinical epidemiology from the University of Pennsylvania where he also completed his residency in Neurology and a Fellowship in Movement Disorders. Dr. Ravina's research interests include developing disease modifying or neuro-protective therapies for neurodegenerative disorders, and the non-motor complications of Parkinson's disease. While at NIH, Dr. Ravina initiated a series of phase II clinical trials aimed at finding disease-modifying drugs for Parkinson's disease. One of the four agents tested will soon be going into a phase III clinical trial. Dr. Ravina completed one of the first clinical trials assessing drugs for the treatment of dementia in Parkinson's disease and was instrumental in developing diagnostic criteria for depression and psychosis in Parkinson's. Dr. Ravina serves on FARA's Board of Scientific Advisors.*



### How did you first get involved in FA?

I got involved in FA research through my colleagues, Kurt Fischbeck and Paul Taylor, who were then at NINDS (National Institute of Neurological Disorders and Stroke). I had worked with them at Penn. Paul and I were residents together and Kurt was our attending. I then got involved in their FA projects and became more interested in this area.

### Tell us about your experiences at the National Institute of Health and NIH's role in facilitating FA research.

At NINDS I worked in the clinical trials group organizing and monitoring clinical trials in many areas but particularly in Parkinson's disease. I was able to add to the FA studies by contributing expertise in the design, conduct, and analysis of clinical trials.

### At the University of Rochester, you are working on coordinating clinical trials for various neurological diseases. What are you currently doing?

I am currently working on several clinical trials in both Parkinson's and Huntington's diseases. These span from drugs slowing the progression of these diseases to ones that may offer short-term symptomatic improvements. Some of the compounds such as COQ10 are similar and stem from a common notion of the role of mitochondrial dysfunction in neurodegeneration. I am also working on studies to identify

and test biomarkers to measure disease progression and enhance diagnostic accuracy for Parkinson's disease.

### The University of Rochester is one of the clinical network sites for FA. Tell us about your plans for FA clinical trials.

We are eager to get our clinical site as active as possible. We are participating in an ongoing natural history study, with Dave Lynch at Penn as the principal investigator, and hope to participate in upcoming clinical trials.

### You will also be coordinating collection of data for FA trials. What's involved in this work?

This starts with the design of a scientifically sound and feasible protocol. The next steps are to select, organize, and train sites on study procedures and measures. Implementing the study requires working with and obtaining approval from regulatory authorities such as the FDA, Health Canada, and other regulatory bodies, depending on where the study sites are. The conduct of any trial involves careful monitoring of safety and collection of data using our web-based system. We have experienced project and data management groups who have conducted many trials. We work closely with the biostatistics department to conduct interim and final analyses. Running a trial successfully requires a multidisciplinary team.

### How do you see FA research and research in other neurological diseases such as Huntington's, ALS, and Parkinson's being mutually beneficial? Do you see the same compounds having treatment benefits across various disease groups?

As I mentioned, there seem to be common themes in many neurodegenerative diseases. In FA, Parkinson's and Huntington's, mitochondrial dysfunction is a common theme and there have been several trials of COQ10-like drugs such as idebenone. More potent compounds in this class are in development and are of interest to researchers in all of these disease areas. Similarly in Huntington's, transcriptional dysregulation (when the gene is not read properly) may play a role and there are ongoing studies of HDAC inhibitors. The overlap of drugs is important in learning about the relevance of the disease mechanisms and if we can share data and experiences across rare diseases like FA and Huntington's, we will have a better chance to explore safety issues and obtain approval of novel compounds.

*Continued on p. 11*

By Pamela Rasey

It has been a wonderful year in development at FARA. We continue to grow our large donor base through talking with individuals, companies and foundations. Since our last newsletter, another \$225,000 has come in through these avenues. This total does not include grassroots fundraising efforts done by families and friends in the FA community.

### Growing Strategies for Success

In the fundraising world, especially when trying to cure a disease, it never seems it can go fast enough. In an effort to increase our speed, we recognize the importance of growing our large donor base. There are a few ways we are working to do just that:

- **Creation of a Development Board** is one way we are updating our structure to add a new team of people. The team will serve on a Board to assist FARA in larger development initiatives by helping us create strategies as well as "ask" for support in their respective network of contacts.
- **New Tools in Place** such as our FARA video, presentations and brochures, the Fundraising Kit and Research Pipeline visually demonstrate where treatments are today. One parent creatively called this "hardcopy hope." More tools will be added in 2008 to give grassroots fundraisers and the Development Board more power to raise money.
- **Regional FARA Representatives** are also another way we are starting to work with FA parents and friends that want to get involved at a higher level in creating awareness and raising money in their respective communities. These representatives work directly with me, as Development Officer, to gain larger donor support.
- **Letter Campaigns and End-of-Year Appeals** are becoming more popular. I am working with several individuals to create letters to key contacts to gain support and possibly extend it to a meeting if a company is interested in looking at FARA as a charity-of-choice or sponsor for specific research/projects.
- **Individual or Private Events** is another area that is surfacing as a way to get businesses involved (as a sponsor) and to grow our network of larger donors. A dinner party, polo party or cruise ship invitations are all ideas that have come up in the last few months. Fundraising is only limited by our imaginations it seems.

Success comes in many forms. We welcome the opportunity to continue to grow the large donor aspect of our fundraising. We work with companies in many

ways through: single or multi-year donations, matching gifts, United Way requests, company sponsorship of an event or project, FARA as a charity-of-choice for specific fundraising events, cause-related marketing, whereby FARA gets a percentage of proceeds from a product sale or customers get asked to "donate a dollar" for a cause, stock donations, in-kind donations and more. ■

Pamela Rasey, Development Officer  
[pamela.rasey@cureFA.org](mailto:pamela.rasey@cureFA.org)  
 513.659.8203

### Giving Options

There are many ways to support the critical work FARA helps to fund. Here are a few examples:

#### Individual Donors

- Yearly donations
- One-time donations
- Stock donations
- Planned giving
- Memorial gifts
- Family fundraisers
- Family foundations

#### Corporate Donors

- 2-3 year donation commitments
- One-time donations
- Stock donations
- Specified grants
- Sponsorships
- Cause-related Marketing
- Matching gift programs
- In-kind gifts

#### Additional Support

- Referrals to other companies or individuals
- Service donations (celebrity tie-ins, marketing or technical services)
- Foundation Grants

### *In Memory...*

We deeply appreciate all of those who think of FARA when they lose a loved one. During 2007, FARA has received \$47,000 in donations in memory of the following individuals:

David McIntyre, Helga Montgomery, David Stambaugh, Paul Robinson, Thomas Barnett, Mary Lou Harrison, George Kobler, Annie Gulliver Reed, Betsey Neyland, Robert A. O'Neil, Sr., Deacon Joseph Orth, Benjamin C. Raymond, Jr., Josh Robiero, Raymond Sojka, Barbara Bucklow, Monica Bruno, Jesse Weaver, Carmen Crimi, Debbie Metz, Scott Gardner, Dorothy Mitchell, and Bobby Wooldridge.

FARA now has envelopes that can be used for this purpose. Please contact FARA at [marilyn.downing@cureFA.org](mailto:marilyn.downing@cureFA.org) if you are in need of the envelopes and we can send them overnight to you.



## Corporate Giving

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)



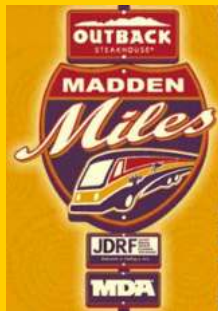
### Company Spotlight

#### Outback Steakhouse Madden Miles Program

This football season, Outback Steakhouse and John Madden have teamed together in the Outback Steakhouse Madden Cruiser to create a program to support two charities: the Muscular Dystrophy Association (MDA) and the Juvenile Diabetes Research Foundation.

Outback is tracking the Madden Cruiser as it travels to football games across the country with the goal of raising one million dollars. All MDA proceeds will go towards FA research.

See <http://www.outbacksteakhouse.com/sportsandentertainment/maddenmiles.aspx>



#### Food Lion Grocery Program

Food Lion has a program designed to allow you to donate a percentage of your grocery costs to FARA. This is done by registering your MVP card. Below are the steps to participate in this program. This will help raise money and it cost you nothing extra.

To enter your MVP Card so that a percentage will go to the FARA fundraising:

1. Go to [www.foodlion.com](http://www.foodlion.com).
2. On the left window pane under *In the Community*, click **Fundraising**.
3. Click **Register MVP Cards**
4. Click the **Select Organization** button
5. In the next window, select **VA** in the state field
6. Select **Springfield** in the city field
7. Select **Friedreich's Ataxia Research Alliance** in the organization field
8. Click **OK**
9. Click **Add MVP Card** button
10. Fill in MVP Card Number and other information required
11. Click **Next**
12. Click **Finished**

Most companies, and people, like to be associated with success. The more businesses we can get interested in working with FARA, the faster research will go. The more the merrier! Imagine being part of helping cure a disease and having a great impact on other neuro-related diseases at the same time. -Pamela Rasey, FARA Development Officer

## A Closer Look at Corporate Support

By Pamela Rasey

Sometimes it's as simple as education. There are many companies out there that give to charities, have a foundation or look for ways to give back. We have only scratched the surface in looking at ways to creatively work in this area to raise money for FA research.

How can we leverage corporate giving?

1. **FA Employers:** We can look at all the companies where FA patients and parents work today as possible supporters in our network. They may be willing to extend support with the right education about FARA. Contact Pamela!
2. **Corporate Programs:** See "Company Spotlight" on Outback Steakhouse's Madden Miles Program as one example.
3. **Think Local:** See the Food Lion "Company Spotlight." Grocery stores and other chains often offer programs whereby a percentage of a purchase can go towards a charity – sometimes it just takes a little research to sign FARA up to be on a list and let people know. We are now in Food Lion's network thanks to FA parent, Terry Benton.
4. **Matching Gifts:** Many companies are committed to supporting their communities, and one way they do that is by matching gifts that their employees make to eligible not-for-profit organizations. This means that a donor's gift could go twice as far. Companies that participate in matching gift programs usually require donors to submit forms to initiate the match. These forms can usually be found in a company's human resources department.
5. **United Way:** We now work with about fourteen UW organizations throughout the United States. Please note that local UW policies vary and you may want to contact your UW yourself to encourage them to allow FARA to be one of their approved charities. FARA will also contact your UW, at your request, to develop a relationship and to get contact Company Spotlights. ■



## Grassroots Fundraising (continued from p. 1)

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)

FARA now is able to offer increased support for the efforts of our “event planners”.

- Fundraising kit
- FARA Brochures, Video and Banners
- Literature for corporate sponsorship
- Mentoring by experienced fundraisers
- Advertising and online donations through the FARA Web site
- Public relations and media advice

There are so many in our “FA family” that have done fundraising, we hope to call upon those of you with experience to offer advice and support to the first-timers. We’ve grown from our first fundraiser in 1999 to a total of 37 in 2007! Approximately \$600,000 has been raised through 2007 grassroots fundraising alone. All of us appreciate those families involved as well as their communities who supported their efforts.

We have an incredible group of people striving to reach our common goal in a variety of ways.

The 2007 events are listed on page 10. Many of these were highlighted in our April and August eAdvocate online at [www.cureFA.org](http://www.cureFA.org). This fall included a variety of events and we are hearing about some unique ideas—a casino night, “Dream Dinners” ([www.dreamdinners.com](http://www.dreamdinners.com) with a percentage of the profits donated to FARA), a kickball game and a quilt raffle.

**Enjoy the pictures from our 2007 events throughout this issue of the Advocate. ■**

### Information Technology Corner

By Jennifer Farmer

The EDS volunteer team continues to bring IT solutions to FARA that contribute directly to advancing treatments for FA and enhance FARA's abilities to achieve our mission faster. Examples include:

#### FA Scales Study

In 2004, EDS completed production of a web-based, HIPAA compliant, system that collects patient information and research data as it pertained to 10 standard tests that are done to monitor the progression of disease. Analysis, scientific computations and reporting capabilities were built into the system which has been in production for over three years. Microsoft, Children's Hospital of Philadelphia (CHOP), EDS, FARA, and Research Scientists from 8 Centers were all participants in the creation of the system and in the success of the research studies. EDS is currently working with the University of Rochester (UR) to import the data from Ataxia Scales study into a UR system

to prepare for coordination of clinical trials.

#### FARA Patient Registry

Global patient database that stores pertinent demographic and medical information that can be used to facilitate recruitment for upcoming clinical trials. The patient registry was a Rochester Institute for Technology and EDS volunteer effort in development and implementation. EDS provides ongoing support of this IT solution on a volunteer effort.

#### FARA Research Portfolio Management Program

Currently there is not an electronic means to manage the submission, review and status for FARA's research grant program. A dedicated team of EDS volunteers have been working for two years to develop a custom web-based solution for FARA to manage all aspects of the grant program. This system will be implemented in early 2008.

#### Virtual Office, Fundraising and Donor Database

Since FARA operations are not centralized to a single location there is a need for a virtual office, a place where FARA officers and staff can schedule meetings, share and manage documents, send messages, and manage projects. Also, there are many new technologies and software solutions to help non-profit organizations with online fundraising and managing a donor database. EDS volunteers have been helping FARA gather requirements and test various solutions in all of these areas.

Everyone at FARA is so grateful to the EDS volunteer team. “Working with these professionals to bring real solutions that accelerate our ability to find treatments for FA has been one of the most inspiring experiences of my career. These people are truly committed to making a difference.” ■

*-Jennifer Farmer, FARA Executive Director.*

**Get Signed Up in the Patient Registry Database for Future Clinical Trials:**

[www.cureFA.org/registry/](http://www.cureFA.org/registry/)

# FARA Fundraising (continued from p. 9)

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)

2007 Events	Event planners	Location	In honor of	Amount raised (approx)
Dream a Little Dream dinner dance	Mary Caruso	Branford, CT	Sam and Alex Bode	\$42,000
Fuzzy Buzzy Golf Tournament	Mary Ann O'Neil, Uncle Paul Staniech	Windham, NH	Erin O'Neil	\$9,200
Canton Fall Classic 5K Race	Christine DeLorenzo	Bridgewater MA	Tiffany Gambill	\$1,300
The Bullpen Open Golf Tournament	Jason Krogmann	Saratoga Springs, NY	Dylan McDonnell	\$4,500
Stars for a Night... A Glimmer of Hope	Dave Brown	Rochester, NY	Alec Brown	\$9,500
Garage Sale	Diane Streuli	Smithtown, NY	Peter Streuli	\$2,400
Westchester Triathlon – Team Donovan	Jennifer Sinnott Simpson family	Rye, NY	Donovan Simpson	\$39,000
Silent Auction, bake sale, restaurant event (Cheeburger, Cheeburger)	Debbie Binko	Tonawanda, NY	Alisa McFarland	\$1,550
Golf	Dan Olsen	Monroe, NJ	Dan Olsen	TBA
Restaurant event and raffle	Julie Cernosek	Arlington, VA	John Cernosek	\$3,000
Kickball game—US Patent Office	Laura Cernosek	Alexandria, VA	John Cernosek	TBA
Charlotte RaceFest Half Marathon	Tracy Rood	Charlotte, NC	Thomas Barnett	\$3,200
Golf and "Funraiser"	Nelda Van Schoick	Bogart, GA	Robbi and Becca Van Schoick	\$60,000
Walk and Restaurant event Loco's Grill	Lindsay Ashman	Savannah, GA	Lindsay Ashman	\$17,220
Car wash	Ruth Magnuson	Pompano Beach, FL	Carly Magnuson	TBA
Family Fun Run	Karla Wooten	Gainesville, FL	Josh Wooten	\$4,400
Event	Bonnie Sibert	Deerfield Beach, FL	Friend's 16 yr. old daughter	TBA
Restaurant event at Max and Erma's	Ashlea Weigand	Robinson, PA	Ashlea Weigand	\$1,510
Charity Backyard Bash	Peter and Francine Welsh	Harrisburg, PA	Brendan Welsh	\$20,480
Picnic, auction	Les Goldstein	Glenview, IL	Alena and Alisa Wolfson	\$17,000
Bake Sale and Chili party	Brad Crosby, Dave Morris	Portage, IN	Thomas Hook	\$6,725
Race for a Cure	Tammy Luebbe	Cincinnati, OH	Evan Luebbe	\$33,500
Miracle event	Edythe Olson	Minnesota	Aubrey and Nicholas Olson	\$11,600
Quilt raffle	Linda Lehn	St. Cloud, MN	Allison Ridgely	TBA
Party, auction	Ruth Neyland	Fort Stockton, Texas	Betsy Neyland, Emily Neyland	TBA
Walk for Hope and A Cure	Sandy Lane	Orange, CA	Chelsea Lane	\$137,000
"Dream dinners"	Debbie Allsopp	Torrance, CA	Crystal Allsopp	TBA
Stephanie's Hope Holiday Boutique	Sharon Magness	Valencia, CA	Stephanie Magness	TBA
California International Marathon	Heather Riggs	Folsom to Sacramento, CA	Friend just diagnosed with FA	TBA
Walk and BBQ	Carrie Laird	Bakersfield, CA	Jerod Laird	\$16,430
Sunset on Friedreich's Ataxia Gala (SOFA)	Paul Konanz, Val Bennett	Alameda, CA	Brianne Konanz and Philip Bennett	\$70,250
Four Bridges Half marathon	Breanne Moen	Folsom, CA	Rachel Gill	\$1,500
Ride Ataxia (Cycling)	Kyle Bryant	San Diego to Memphis	Kyle Bryant	\$42,700
BRO Golf in memory of "Rosie"	Peter Bugnatto	Vancouver, BC, Canada	"Rosie" Rosencranz	\$11,000
Calgary Marathon	John Luth	St. Albert, Alberta, CANADA	Joel Kleine	\$2,130

\*TBA—Last quarter events. Amounts to be announced.

**See Page 13 for Upcoming Fundraising Events!**

## Featured Scientist (continued from p. 6)

## FARA Officers & Directors

### What do you see as the most promising FA research currently underway?

There is a lot of exciting work in FA and I could not choose one area. It is hard to predict success in patients based on success in the lab. In reality the more interventions that we are able to test in patients, the more likely we will be to find an effective treatment. The key is to make this translational process, from preclinical to clinical trials, as efficient as possible and ensure that each trial adds important information that will allow us to decide if an intervention should be pursued further or should be abandoned.

### How long do you think it will be before there is an effective treatment for FA?

I think FA is poised for real progress and can be a model for targeted interventions in neurodegenerative diseases. It is hard to know how long this will really take but there is good reason to be optimistic. ■

*We would like to recognize donors that have given \$10,000 or more in 2007. FARA thanks you for your continued support.*

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## Ride Ataxia II

Visit our Web site at [www.cureFA.org](http://www.cureFA.org)

By Kyle Bryant

Last year Kyle and his recumbent tricycle were joined by his dad, Mike, his mother, Diane, his uncle, Steve, fellow ataxian David "Spinner" Henry, his elementary school friend, Andy, and other dedicated supporters for the 2,400 mile ride from San Diego, CA to the National Ataxia Foundation's annual conference in Memphis, TN to raise awareness and research funds for Friedreich's ataxia research. The event was a HUGE success! Despite knee injuries, wind storms, freezing temperatures and rotten road kill, the team visited FA researchers and patient families along the way, and made it to Memphis on time and in good health.

The team raised over \$40,000 and even more exciting was the uniting of the National Ataxia Foundation and the Friedreich's Ataxia Research Alliance for the first time ever. Both of these organizations contributed matching funds to create the \$100,000 Kyle Bryant Translational Research Award. By June 2007, the grant was awarded to Dr. Nuri Gueven at Queensland Institute of Medical Research in Australia for promising FA research.

The Ride Ataxia Team is at it again! The team will be cycling 620 miles from Sacramento, CA (Kyle's hometown) to Las Vegas, NV starting March 15 and ending on March 27 at the Flamingo for the NAF Annual Membership Meeting. Moving at a pace of about 50 miles a day, the ride will be fully supported with vehicles, food and accommodations. The team welcomes anyone who is up for the challenge. Here are the basics:

- You can join for all or part of the ride.
- Ride Ataxia is asking cyclists to raise at least \$1,625 or \$125 a day toward the research funding goal.
- For more details check out <http://rideataxia.org>, <http://rideataxia.blogspot.com> or e-mail [kyle@rideataxia.org](mailto:kyle@rideataxia.org)

We can't wait to get on the road to a treatment and a cure!

See you in Vegas! ■



*Kyle Bryant with his uncle Steve Bryant, father Mike Bryant, friends Mary Krill and Wally Krill, and mother Diane Bryant*



*Kyle Bryant (center) with his father Mike Bryant (left) and uncle, Steve Bryant (right)*

Get Signed Up in the Patient Registry Database for Future Clinical Trials:

[www.cureFA.org/registry/](http://www.cureFA.org/registry/)

## Upcoming Events

To build on the successes of 2007, our goal is to increase the number of events in 2008. This issue of the Advocate is reaching an audience of over 2,000 homes. If you have FA or someone you care about has FA, please consider being an active part of this wonderful cause. Keep the momentum going by planning a fundraiser in your community. We are so close to getting our treatment.

Here is a sampling of ideas to get you started:

- Consider starting with a letter writing campaign (see [www.cureFA.org](http://www.cureFA.org) for samples)
- Contact a local restaurant to see if they will donate a percentage of sales on a specific night—then round up your friends and family!
- Look at the list of upcoming events for 2008 and get involved with an event in your general area.
- Download or contact FARA for the Fundraising Kit.
- Perhaps hold an event in conjunction with the Friedreich's Ataxia Awareness Day, Saturday, May 19, 2008 or International Ataxia Awareness Day on September 25, 2008.

Contact the chair of our fundraising committee, [marilyn.downing@cureFA.org](mailto:marilyn.downing@cureFA.org), to complete a fundraising form when organizing your event for 2008. Below is a list of a few of the upcoming events for 2008. ■

2008 Events	Event planners	Location	Contact Info	Date
Leaping for a Cure (Casino night)	Karla Wooten	Gainesville, FL	<a href="mailto:karla.wooten@sfcc.edu">karla.wooten@sfcc.edu</a>	February 29, 2008
The Voyces Concert	Brian Wurschum	New York City	<a href="mailto:thevoyces@aol.com">thevoyces@aol.com</a>	Feb/March 2008
Ride Ataxia II (Cycling)	Kyle Bryant	Sacramento to Las Vegas	<a href="mailto:bryant.kyle@gmail.com">bryant.kyle@gmail.com</a>	March 2008
5K, 10K and BBQ	Carrie Laird	Bakersfield, CA	<a href="mailto:keyedup@bak.rr.com">keyedup@bak.rr.com</a>	April 6, 2008
Golf tournament and "Bash"	Nelda Van Schoick	Bogart, GA	<a href="mailto:neldasvs51@yahoo.com">neldasvs51@yahoo.com</a>	April 25, 2008
Race for a Cure	Tammy Luebbe	Cincinnati, OH	<a href="mailto:tammyluebbe@aol.com">tammyluebbe@aol.com</a>	May 20, 2008
Golf tournament	Rick Peters	Peoria, IL	<a href="mailto:rick@callenderco.com">rick@callenderco.com</a>	May 29, 2008
FA in the Heart (FAITH)—dinner gala	Bart Rupel and family	Santa Clara, CA	<a href="mailto:bartrupel@yahoo.com">bartrupel@yahoo.com</a>	May 31, 2008
Event	Kathy Smith	Carencro, LA	<a href="mailto:KPSmith80@cox.net">KPSmith80@cox.net</a>	Mid-June 2008
Stephanie's Hope Dinner event	Sharon Magness	Valencia, CA	<a href="mailto:cnsmags@yahoo.com">cnsmags@yahoo.com</a>	Summer 2008
BRO Golf Tournament	Armando Martinez	Washington DC	<a href="mailto:amartinezcpa@gmail.com">amartinezcpa@gmail.com</a>	Summer 2008
Dinner dance	Mary Caruso	Branford, CT	<a href="mailto:meirbode@aol.com">meirbode@aol.com</a>	September 2008
Fuzzy Buzzy Golf Tournament	Paul Stanieich	Windham, NH	<a href="mailto:MDay485@aol.com">MDay485@aol.com</a> (Mary Ann O'Neil)	September 2008
5 <sup>th</sup> annual Golf Tournament	Dan Olsen	New Jersey	<a href="mailto:dolsen@pmcink.com">dolsen@pmcink.com</a>	September 2008
Stephanie's Hope Holiday Boutique	Sharon Magness	Valencia, CA	<a href="mailto:cnsmags@yahoo.com">cnsmags@yahoo.com</a>	November 2008
Party, auction	Ruth Neyland	Fort Stockton, TX	<a href="mailto:ruth_neyland@hotmail.com">ruth_neyland@hotmail.com</a>	December 17, 2008

**Request a Fundraising Kit**  
<http://www.curefa.org/fundraising/>





*The Bro Golf Tournament golfers together in memory of "Rosie"*



*The team of Ryan Platt, Breane Moen and Mackenzie Morgan run in honor of their friend with FA*



*Tiffany Gambill with runners Julianne Benive and Laura DeLorenzo*



*Employees of the US Patent and Trademark Office in Virginia play kickball to raise funds for ataxia research in honor of John Cernosek*

*The FARA Advocate is brought to you by:*

*Editors: Ron Bartek, Marilyn Downing, Jennifer Farmer, Mary Beth Kosmicki, Paul Marcotte, Pamela Rasey*

*Advisor: Bill Hartnett*

*Design/Layout: Meg Giaconia*





*Lined up and ready to roll at Luebbe event in Cincinnati*



*FA parents at Stars for a Night. Karen Brown, Marilyn Downing, Bob and Margaret Ferrarone, Mary Caruso, Terry Downing, Lori Siracusa, Dave Brown, Ron Bartek*



*Alex Bode (left), Paige Lane and Allison DePaola at the Caruso/Bode event*



*Eilish taking care of the raffle tickets with mom, Francine, supervising at the Welsh backyard bash*



*Alec Brown sings at the Stars for A Night... A Glimmer of Hope event in Rochester*



*John Levis performs at the Welsh backyard bash*





*Marianne Cernosek and son John enjoy happy hour in his honor sponsored by the Front Page and WASH 97 FM*



*The Cernosek event in Arlington included a raffle of cosmetic services, image consultation and golf packages*



*Eager volunteers at Wooten event (who says it never rains in Florida?)*



*Josh Wooten partying at his family's well-supported fundraiser in Gainesville, FL*



*Julie Cernosek (and generous friends) at the Front Page happy hour in Arlington, VA*





*Phillip Bennett at the Sunset on Friedreich's Ataxia Gala*



*Tracy and Jamie Rood run to honor friend Thomas Barnett (NC)*



*Ashlea Weigand feels the love at her event at Max and Erma's*



*Les Goldstein, the organizer of the Chicago area fundraiser in honor of the Wolfson family, gets soaked!*





*FARA president Ron Bartek joins the Van Schoicks at their 2007 Georgian golfing event.*



*Ian Pound from New Hampshire is in California to help his cousin Chelsea Lane*



*The Lane and the Magness families at the Lane's very successful Walk for Hope and a Cure*



*Chili Party in Portage, IN in honor of the Hook family*



*Go, Team Donovan! Some of the athletes participating in honor of Donovan Simpson in Westchester, NY*





*The Bullpen Open golfers enjoy a beautiful autumn day in Saratoga Springs, NY*



*Samantha Bode with Matt Hill at Dream a Little Dream (CT)*



*Erin O'Neil hitches a ride at the Fuzzy Buzzy Golf Tournament*



*Live auction at the Sunset on Friedreich's Ataxia event in Northern California*



*Dyan McDonnell's mom, Terri (in back) with Kristine Orr in Saratoga*



*An evening at the Rosenblum Cellars (Sunset on Friedreich's Ataxia)*

## Contact Us



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