

# The Advocate

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



Issue #8

Winter 2008-2009

## Clinical Trials Update

### Friedreich's Ataxia Treatment Pipeline

By Jennifer Farmer

FARA is excited to report on the promising clinical trials that are currently under way and anticipated in the coming year. In 2008, there have been three clinical trials in FA patients and one trial of a new drug for FA in healthy humans. A brief summary of 2008 FA clinical trial accomplishments include:

#### 2008 Accomplishments

- A phase III clinical trial of Idebenone was launched at the Children's Hospital of Philadelphia (CHOP) and the University of California Los Angeles (UCLA) by Santhera Pharmaceuticals. Study enrollment was completed at the end of October 2008 with about 70 patients. The FARA Patient Registry was used to help recruit patients for this study. Thank you to all of the study participants – success in clinical trials depends directly on your involvement.
- The Canadian regulatory agency, Health Canada, licensed a new formulation of Idebenone, Catena, by Santhera Pharmaceuticals for treatment of FA. This is the first approval of a drug for treatment of FA. Catena (Idebenone) is an antioxidant that is similar to co-enzyme Q10. It is a small molecule that facilitates the transport of electrons within mitochondria, which is necessary for the production of cellular energy.
- A Phase I/II study of Deferiprone, an iron chelator, was launched in Europe (France, Italy and Belgium) by Apopharma based on very promising results in a pilot study conducted in France published last

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## EDS Awarded FARA Partner of the Year

By Jennifer Farmer

FARA's mission and vision clearly state our core belief in the power of partnership and collaboration. In 2008, FARA therefore launched a recognition program in which we identify and recognize one partner each year to highlight as "FARA Partner of the Year."



*EDS Volunteers: Rich Duse, Margaret Ferrarone, Marty Ohman, John Jones, Marianne Wilcox, Mary Beth Kosmicki, Barbara Kassnoff and Bill Hartnett.*

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### What is Friedreich's ataxia?

Friedreich's ataxia is a debilitating, life shortening, degenerative neuro-muscular disorder. Onset of symptoms can vary from childhood to adulthood and can include loss of coordination (ataxia) in the arms and legs; energy deprivation and muscle loss; vision impairment, hearing loss, and slurred speech; aggressive scoliosis (curvature of the spine); diabetes mellitus; and a serious heart condition. Childhood onset of FA is usually between the ages of 5 and 15 and tends to be associated with a more rapid progression. Late onset FA (LOFA) can occur anytime during adulthood. While the mental capabilities of people with FA remain completely intact, the progressive loss of coordination and muscle strength in FA leads to motor incapacitation and the full-time use of a wheelchair. There is currently no treatment or cure for FA. FARA is a 501 (c) (3) tax exempt, non-profit organization dedicated to supporting research that will improve the quality and length of life for those diagnosed with Friedreich's ataxia and will lead to treatments that eliminate its symptoms.

## President's Message

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



Dear Friends and Partners,

With three clinical trials in FA underway and several more being prepared, FARA has been fortifying its Board of Directors and Scientific Advisory Committee with dedicated people possessing the additional capabilities, experience and expertise needed to help steer FARA most effectively through this "clinical era" and accelerate us on the path to treatments and a cure. During the last year, FARA's Board of Directors has elected its first chairperson and has voted to add a number of new members.

FARA recently elected Dr. Tom DeCotiis to be its first Chairman of the Board. Tom is co-founder and CEO of Corvitus, a company providing high-level management consulting services to a wide range of companies. He received his PhD in industrial and organizational psychology from the University of Wisconsin and has served on the faculties of Cornell University and the University of South Carolina. Tom has already contributed immensely to FARA's mission, and we are excited and privileged to have Tom leading FARA's Board of Directors.

FARA's most recently elected members of the Board of Directors are:

**Dr. Earl Giller** -- M.D., PhD in Neurochemistry; Global leader in drug and biomarker development while at Pfizer; Former VP for Clinical Development at Marinus Pharmaceuticals; Senior Scientific Advisor with MedAvante; Independent consultant on neuro-drug development.

**Peter Pitts** -- President, Center for Medicine in the Public Interest; Senior Vice President/Global Health Affairs/Manning, Selvage & Lee; Former Associate Commissioner/U.S. Food and Drug Administration.

**Laura Kalick** -- Attorney at Law; In-depth experience at IRS, U.S. Senate staff, and large accounting firms; Specialist in non-profit organizations and their tax status, Assisted FARA with its initial submission to the IRS for non-profit status and throughout our ten years of operation.

**Nancy Schneid** -- Corporate marketing and branding advisor; elected to Advertising Age's "Marketing 100;" Former EVP and Chief Branding Officer of OSI Restaurant Partners; Board of Trustees of University of

South Florida and St. Joseph's/Baptist Health Care; Board of St. Joseph's Hospital Foundation.

**J. Ed Ramsey** -- Co-owner/VP of Taylor-Ramsey Corp. (lumber products) and Head of International Trade Division; VP of BEPCO (timber & land development); Chairman of Appalachian Hardwood Manufactures, Inc.

In addition to reinforcing the Board of Directors to navigate the "clinical era," FARA has taken on a Vice President/Chief of Science to help take full advantage of the rapid and significant growth in scientific investigation of FA. FARA is proud and fortunate to announce that Dr. Giovanni Manfredi, M.D., PhD, Professor of Neurology and Neuroscience at Weill Cornell Medical College, has agreed to serve in this new capacity. Dr. Manfredi's FARA responsibilities include chairing the Scientific Advisory Committee, overseeing the research process and portfolio, identifying and filling any gaps in that portfolio and serving as scientific liaison between FARA and MDA. He will also be FARA's ambassador to the broader scientific community -- looking to bring additional perspectives, insights and talents to our FA cause.

FARA is confident that all of these wonderful additions to the FARA team will help in moving us more quickly to treatments and a cure. We are certain, though, that FARA will be successful only with your support. All the new talent will help FARA navigate the "clinical era" but the all-important clinical trials will succeed only if more and more FA patients are enrolled in FARA's patient registry and are prepared to participate in those trials. Also, FARA relies on the committed efforts of its grassroots fundraisers- FA families across the country who host events and inspire giving to support FA research efforts in their communities. This year FA research benefited from the continued success of our veteran fundraisers and a growing number of first time fundraisers. We encourage everyone to consider how they might organize in their own community or support existing fundraising activities.

Each one of us can make a difference. Together, we will make THE difference.

Warm regards,  
Ron

year. Enrollment is open. Study participants are needed; however, you must be a resident in the country of the host site. Additional study sites are anticipated in Spain, Canada and Australia. Visit the FARA Patient Registry and Clinical Trials Web site for site contact information: <http://www.curefa.org/trial.html>

- Erythropoietin (EPO) is a hormone produced in our bodies and is also an approved drug used to increase red blood cells. It is commonly used in dialysis and cancer patients as well as in patients just prior to surgery in which loss of blood is anticipated. Drs. Scheiber-Mojdehkar and Sturm found that EPO increased frataxin levels in laboratory cell models, and in 2008 with Dr. Sylvia Boesch they completed a six month open-label clinical pilot study of safety and efficacy of rhuEPO treatment in FA. This was a small study with eight adult FA patients. They reported that frataxin levels were increased in patients and measures of oxidative stress were reduced. There was also some evidence suggesting clinical improvement based on neurological rating scales. Half of the patients in the study did have elevations of hematocrit (a red blood cell measurement) that required phlebotomy which is a significant safety concern.
- A0001, a compound discovered by Edison Pharmaceuticals that shows promise of improving mitochondrial function (energy production) in FA patients and other patients with mitochondrial dysfunction disorders, has been advanced to human safety trials by Penwest Pharmaceuticals. In July 2008, Penwest launched a Phase Ia safety study of A0001 in healthy humans. A second Phase Ib study to determine dosage is anticipated to follow upon completion of Ia. These studies are necessary to determine safety and dosage, because this is a new drug. Then studies in patients can be properly planned and implemented. Many new drugs take years (5-10) of development before getting to human trials. A0001 has moved from discovery to human trials in about 2.5-3 years.

Thanks to the brilliant and committed efforts of many FA scientists and our drug development partners, we are now seeing novel drugs advance through development to patients. We believe that research and drug discovery requires a diverse and strategic approach. Throughout FARA's research pipeline, there are other early stage studies that are designed to investigate new treatments. We believe that we need multiple drugs in each target area to ensure successful treatments are delivered to all patients with FA. We know that we are not going to be able to find effective treatment with just a single drug or intervention. FARA and FA scientists remain confident that these clinical trials will result in the first approved treatments for FA and will begin building the combined or "cocktail" therapy.

Based on the momentum of research in 2008, with the discovery of new drug targets and advancement of drugs through development, we anticipate several new clinical trials in 2009. The HDAC inhibitors, originally discovered for use in FA by Dr. Joel Gottesfeld of Scripps Research Institute, and are being developed by Repligen Corporation, and should be ready to go forward to early Phase I studies in humans. The HDAC inhibitors act at the DNA/gene level and increase frataxin in cells from FA patients and in FA animal models. Also, based on the results of Phase I studies of A0001 described above we hope that clinical trials in FA patients could begin in mid-to-late 2009. There are several new discoveries and additions to our research pipeline (as depicted in the graph) and we'll look forward to sharing progress updates in 2009.

**Your participation is critical.** Please ensure that FA patients sign up on FARA's Patient Registry ([www.curefa.org/registry](http://www.curefa.org/registry)) so they can be notified regarding clinical trials for which they appear to be eligible. This Web site also contains postings and information on clinical research studies. Our drug development partners and clinical research network physicians have told us that patient participation in clinical research studies are absolutely necessary for success, and they are looking to us for that support. ■

*Clinical Trials continued on p. 4*

## Ataxia Research Conference in Dublin

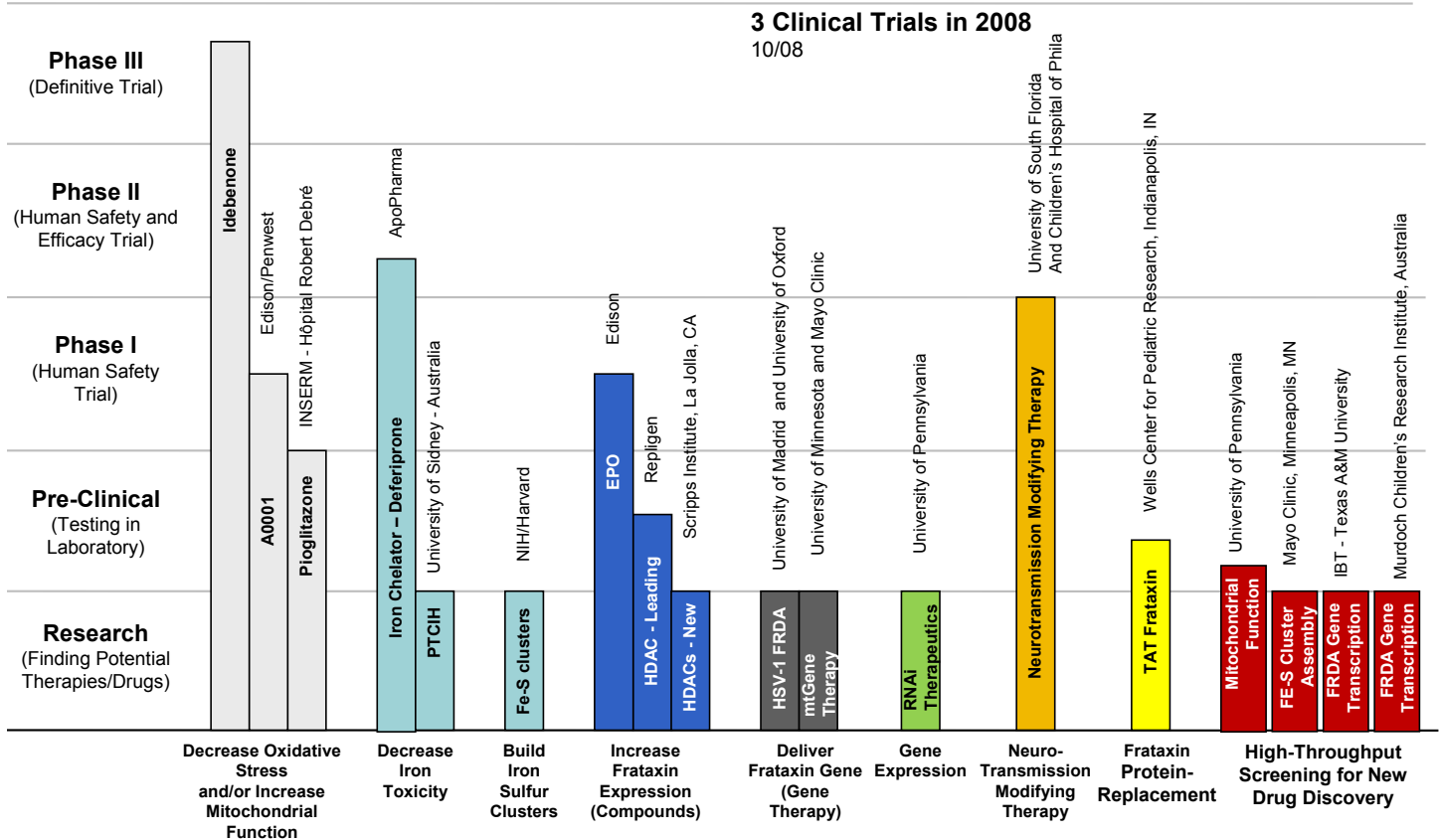
by Dr Julie Greenfield, Ataxia UK

On the 25th of September, coinciding with International Ataxia Awareness Day, a research conference on the ataxias was held in Dublin. This was jointly organized by the Friedreich's Ataxia Society of Ireland (FASI) and Ataxia UK, charities providing support to people with ataxia as well as funding research. Over 100 delegates gathered to hear many fascinating presentations from some of the most eminent researchers in the field. We were pleased to hear talks on the latest research on the promise drugs such as HDAC inhibitors are showing in switching the Friedreich's ataxia gene on, by U.S. researcher Joel Gottesfeld and by Richard Festenstein from the UK. We were also pleased to welcome Piyush Vyas from Indiana University who spoke about delivering TAT-conjugated frataxin to cells and Martin Delatycki who spoke about rating scales and his Friedreich's ataxia specialized clinic in Melbourne, Australia. Dolores Cahill, a researcher from Dublin who works closely with FASI, presented some of her work on proteomics. Massimo Pandolfo gave an excellent update on trials in FA, with an emphasis on the idebenone trial. Santhera's Pierre Vanken was in the audience and was able to provide an update on the status of the trial and market authorization in the various countries. Finally, the Friedreich's ataxia session was concluded by a presentation from Arnold Munich on the ongoing deferiprone trial. The remainder of the conference focused mainly on research into the cerebellar ataxias. A full report of the conference will soon be available on the Ataxia UK Web site [www.ataxia.org.uk](http://www.ataxia.org.uk)

## Clinical Trials (continued from page 3)

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

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



## Attention FA Researchers:

FARA has posted new grant opportunities and guidelines on the FARA Web site: <http://www.curefa.org/grant.html>

Also, there is a new grant submission and management system. All grant proposals must be received through this new online program: <http://www.curefa.org/RPMP/public/pgrpmphome.aspx>

If you have questions, please contact:  
Jen Farmer, [jen.farmer@curefa.org](mailto:jen.farmer@curefa.org)



## FARA Funds 20 Grants Exceeds \$1 Million in Research Dollars

By Jennifer Farmer

The FARA Grant Program has funded 20 grants totaling over \$1 million in direct support for research from January – October 2008. We do anticipate funding some additional grants in the last quarter of the year that will bring us close to \$1.5 million in research funding for 2008.

### FARA's Grant Program Priorities

- Advance drug discovery and have a diverse treatment pipeline
- Facilitate the drug development process and translational research so that the most promising discoveries are rapidly brought to treatment trials
- Support the clinical research, infrastructure and biomarker discovery required to ensure effective and efficient clinical trials in a rare disease
- Support young/new researchers with innovative ideas and a commitment to FA research
- Reduce the morbidity and mortality caused by cardiac disease in FA

Spotlight on exciting projects co-funded with some of our research partners:

- FARA partnered with the American Heart Association to co-fund research that would further our understanding of cardiac disease in FA and related treatments. This year's award recipient was Dr. Mark Payne from Indiana University. Dr. Payne's project is designed to test the hypothesis that a novel therapeutic protein, TAT-Frataxin, can provide enzyme replacement therapy to correct the cardiomyopathy of Friedreich's Ataxia (FA). In preliminary studies with mice conditional for loss of frataxin in brain and heart, TAT-frataxin administration lengthens their lifespan. Dr. Payne will test the hypothesis that TAT-Frataxin increases the lifespan of these animals by reducing the hypertrophic cardiomyopathy associated with FA. These experiments will support the development of a novel therapeutic for FA using protein replacement therapy.
- FARA and the National Ataxia Foundation were able to partner with Ride Ataxia to expand funding for two translational research awards in 2008. This year's award recipients are advancing two very important projects. One award went to Dr. James Rusche at Repligen Corporation and his collaborators who are advancing the development of HDAC inhibitors as a novel therapeutic for Friedreich's ataxia. HDAC inhibitors have demonstrated great promise for increasing the amount of frataxin in the laboratory. Because these are novel drugs, extensive development is required. The objective of this grant is to use model HDAC inhibitors to create a standard measure of frataxin gene expression. These studies will establish quantitative measures of drug in brain tissue of transgenic mice and cor-

relate that to the amount necessary to get more frataxin protein. These techniques can then be used to identify the best compound for development. The second award went to Dr. Subha Raman at Ohio State University who proposed using cardiac MRI to detect and treat FA-related heart disease in its earliest stages. Patients with Friedreich's ataxia (FA) develop weakness of the heart muscle that can ultimately cause death due to abnormal heart rhythms (arrhythmias) or congestive heart failure. This heart muscle disease, also known as cardiomyopathy, may be difficult to treat in advanced stages. Earlier detection and better understanding of why it develops could help create new prevention and treatment strategies that reduce death and disability in FA patients.

- FARA and the Muscular Dystrophy Association partnered to co-host a special mitochondria – focused scientific summit. The groups also issued a request for applications to fund a grant focusing on critical issues or hypotheses that advance therapeutic approaches to mitochondrial dysfunction as it relates to neurodegenerative diseases. The recipients of this award are Dr. Michael Koob and collaborator Dr. Grazia Isaya. Dr. Koob has developed technology that allows him for the first time to engineer mammalian mitochondrial genomes and reintroduce these genomes into mouse embryos. He proposes to use this technology to develop a gene therapy for Friedreich ataxia (FRDA). Initial efforts will focus on correcting the molecular deficits associated with the complete loss of frataxin in the yeast model. They will use the information and reagents developed in the yeast system to move to the mouse models of FA. This experimental system will allow optimization of both the mitochondrial transfer technology and the mitochondrial frataxin gene constructs. Once they have completed this work they will be in an excellent position to develop a gene therapy approach for mouse models of FRDA and to work towards adapting these therapies to treating FRDA and other mitochondrial diseases in humans.
- FARA and FARA Australia were able to partner to fund research on hearing loss in FA, which has been an underappreciated symptom in FA. Dr. Gary Rance in Australia and Dr. Thierry Morlet in the United States have developed a comprehensive protocol to study auditory function in FA patients and to determine if auditory measures can be developed as biomarkers or clinical outcome measures. This research is being done in collaboration with FA Clinical Network sites in both the United States and Australia. ■

**See Grants Awarded in 2008 on p. 6**

Principal Investigator	Project	Research Area	Category	Outcome
Sidney Hecht	Analysis and restoration of Mitochondrial Function	Basic	Mitochondrial	↓ 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
<b>2008 Mitochondrial Therapeutic Approaches Research Award</b>				
*Michael Koob	Towards Gene Therapy of Mitochondrial Diseases	Basic	Mitochondrial	↑ frataxin
Filip Lim & Richard Wade-Martins	Neuron-validated approaches for developing Friedreich's ataxia therapeutics	Basic/ Translational	FRDA Gene	↑ frataxin
<b>2008 FARA New Investigator Award</b>				
Alain Martelli	Iron Metabolism in FA: from the fundamental understanding of iron dysregulation to the therapeutic potential of iron chelators	Basic/Translational	Iron Metabolism	↓ oxidative stress and ↑ mitochondrial function
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
<b>2008 American Heart Association/Friedreich's Ataxia Research Alliance Cardiology Research Award</b>				
%R. Mark Payne	Use of TAT-Frataxin to Reverse the Cardiomyopathy of Friedreich's Ataxia	Basic/ Translational	Frataxin delivery Cardiac	↑ frataxin
Jackson Laboratories	FA Mouse Models	Translational	Animal Models	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
*Grazia Isaya	Modulators of frataxin assembly: assay development for high throughput screening	Translational	Drug Screening	All
<b>2008 Kyle Bryant Translational Research Award</b>				
#James Rusche	Development of Methods to Determine the Pharmacodynamic Effect of HDAC Inhibitors on Frataxin Expression	Translational	FRDA Gene	↑ frataxin
<b>2008 Kyle Bryant Translational Research Award</b>				
#Subha V. Raman, MD and Roula al-Dahhak	Preclinical Markers of Cardiomyopathy in Friedreich's Ataxia	Translational	Cardiac, Biomarkers	All
David Lynch	Mitochondrial protein levels as biomarkers in Friedreich Ataxia	Translational/Clinical	Biomarker	All
Collaborative Clinical Research Network	Mortality in Friedreich's Ataxia	Clinical	All	All
*David Lynch	Collaborative Clinical Research Network for Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
Bernard Ravina	Supplement Clinical Research Network for Friedreich's Ataxia – Data Coordination Center	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
Bernard Ravina	EPO Planning and Implementation Grant	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
Theresa Zesiewicz	Double-blind, Randomized, Placebo-controlled pilot study in the Treatment of Friedreich's Ataxia	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
^Thierry Morlet and Gary Rance	Development of Auditory Biomarkers in FRDA	Clinical	Clinical Outcome Measures, Biomarkers and Trials	All
John Day/ National Ataxia Foundation	Ataxia Investigator's Meeting, LasVegas, NV, March 2008	All	All	All

\* =Matching grant provided by MDA, # = Matching grant provided by NAF, ^ =Matching grant provided by FARA-Australia, % =Matching grant provided by AHA.

"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 (↑).

## Featured Scientist

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

### Dr. David R. Lynch

Interviewed by Paul Marcotte



*David R. Lynch, M.D., is an Associate Professor of Neurology and Pediatrics. As both a neurologist and scientist, Dr. Lynch takes on dual roles at the University of Pennsylvania and the Children's Hospital of Philadelphia. Dr. Lynch initiated the important, ongoing clinical studies that measure the status of FA in a patient and tell us about the natural history of the disease. He is also the Principal Investigator of the Collaborative Clinical Research Network in FA- a 10 center international consortium dedicated to providing the best clinical care to individuals with FA and implementing clinical research and trials. Dr. Lynch has applied his clinical and scientific experience and knowledge to FA as a FARA advisor – reviewing grants, advising FARA on how to prioritize precious research resources, participating in outreach efforts to educate the greater medical community, advising new investigators, and fostering collaborations within the FA scientific community.*

#### **How did you first get involved in Friedreich's Ataxia Research?**

Kurt Fischbeck and I had followed a moderate size cohort of patients with FA in the early 1990's. Around 1996 Rob Wilson appeared with a project on iron levels in FA that needed a clinician to organize the patient cohort. As a basic scientist by training, I thought it would be interesting to do a small clinical project as well. It worked out well and we have kept working on FA for the next 12 years (with help from lots of people along the way)—the projects aren't small anymore.

#### **When did you first see an opportunity for clinical trials for Friedreich's ataxia?**

One thinks of the potential for well-directed clinical trials as soon as the genetic mutation was identified, and then later once we had some sense of what frataxin does. We started with discussions about a single compound and through years of development at all levels have seen it grow to many possibilities.

#### **What were the initial roadblocks that had to be overcome before starting clinical trials?**

Initially we all thought that when we know what causes the disease, we should have a drug for it tomorrow. But we lacked many things—an appreciation of the need for safety data, ways to measure drug response, methods to identify a large number of affected patients with this disorder in order to get sufficient enrollment, and the need to identify physicians and locations interested in participating. It is no small feat that we have put these things together over the past eight years. Re-

gardless of the outcome of any ongoing trials, these are hurdles we have overcome to a substantial degree so that the next agent (whatever it may be) enters clinical trials faster than it would have 10 years ago.

#### **What are you currently working on?**

Right now, we have about 8-10 ongoing clinical research protocols on FA at our institution. Most of them are natural history studies—studies that allow us to better understand how this disease evolves and how it affects people. These are quite important because they tell us how to measure change in FA and what things truly affect patients' lives. We also have studies looking at biomarkers of disease to assess which blood tests or other biological tests might be used to readily assess response to therapeutic agents.

Finally, we are one of the two sites in the Phase III trial of Idebenone, the trial that will assess whether high dose Idebenone improves neurologic function in FA. Along with safety, this assessment of whether it improves function (and not just markers of disease) is the critical assessment in Phase III for whether a drug is efficacious and thus able to be submitted to the regulatory agencies for approval.

#### **What are your expectations for clinical trials in the U.S. and internationally in the next few years?**

In the upcoming years we will see more trials at every level—the development of diverse approaches of the last 10 years is responsible for that. We have a variety of leads that are promising—therapies for raising frataxin levels, for improving mitochondrial function, and potentially therapies directed at individual components of FA (cardiomyopathy etc). What is unique about FA (for a rare disease) is that we have all of these potential therapeutic strategies—this is what leads to my optimism. Some of the approaches will fail for reasons we don't yet know—that is the reality of drug development. But some of them likely will succeed.

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

I think treatment for FA will evolve over years as some of the proposed therapies are shown to be efficacious. There will probably be multiple therapies that appear over time. If Idebenone is shown to be efficacious in the Phase III trial, it would constitute the first component of such therapy. Thus now is a time where we may be seeing the initiation of treatments that will improve function in FA or alter its long term course. ■

## FA Families Featured in Emmy Winning Program - Keeping Kids Healthy

Keeping Kids Healthy, produced by Montefiore Medical Center in association with Thirteen/WNET and nationally syndicated by American Public Television, won a 2008 New York Emmy for their segment on Friedreich's ataxia. This program features interviews with several FA families (Bode/Caruso, Orth, and Simpson) and can be viewed online through the following link: <http://www.keepingkidshealthy.org>

## Shop to Cure FA

FARA has partnered with Alliance Charities, a non-profit organization, to make charitable giving a simple process. Alliance Charities has secured arrangements with Google and with hundreds of online stores like Amazon.com, Wal-Mart, Target, Disney and many other major retailers to offer Cash Rebates for searching and online purchases. When you enroll as a Preferred Buyer, every qualified search and online purchase made through this site earns money that is donated to FARA on your behalf. Visit [www.shoptocureFA.org](http://www.shoptocureFA.org) to register and start shopping.

## Ride Ataxia III Registration

Portland, Oregon to Seattle, Washington  
March 16-19, 2009

Visit [www.rideataxia.org](http://www.rideataxia.org) for the Ride application and further information.

## BGC Partners, Inc. Selects the Friedreich's Ataxia Research Alliance as a Charity Day Recipient and FARA Raises Awareness about FA During WFAN Radiothon

By Felicia DeRosa

BGC Partners, Inc. selected the Friedreich's Ataxia Research Alliance (FARA) to be one of their 2008 Charity Day recipients. BGC Partners, Inc. (Nasdaq: BGCP) is one of the world's leading inter-dealer brokers. The company was formed as Cantor Fitzgerald rebuilt after the World Trade Center attacks, and was separated from Cantor in 2004.

BGC's Charity Day was originally conceived as a way to raise money for the Cantor Relief Fund, by donating a full day's global brokerage commissions to the Fund. Today the event has evolved and funds raised now also go to dozens of other worthy causes around the world.

In 2007 BGC raised over \$6 million globally. In New York the event is held in their Wall Street offices, with invited celebrity guests acting as brokers for the day. With the guidance of BGC's licensed brokers, they carry out actual trades of bonds, foreign exchange and other financial instruments with BGC clients - Wall Street's biggest banks. On September 11, 2008, Sean Landeta- retired NY Giants punter, Tiki Barber, retired NY Giants running back and current Today Show correspondent, and Rich Monte-leone, NY Yankees Special Pitching Instructor represented FARA on the trading floor for Charity Day.

Broadcasting from BGC's trading floor, WFAN Radio held its annual Radiothon in tandem with BGC's Charity Day. COO of OSI International and FARA Board Member, Paul Avery, had a guest spot on Boomer Esiason's *Boomer and Carton Morning Show* to raise awareness of Friedreich's ataxia and the kind of research FARA supports. ■



## FARA-MDA Summit and Grant

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

### FARA-MDA Summit and Grant Cap FA Mitochondria Year

By Ron Bartek

In our concerted effort to develop treatments and a cure for FA, it is essential that FA scientists and drug companies understand as much as possible about mitochondria – the energy factories of our cells – and about the mitochondrial dysfunction involved in FA. 2008 has been a red-letter year for advancing such understanding.

On May 20-21, FARA and MDA co-hosted a Mitochondrial Summit at Arizona State University in Tempe. A Scientific Steering Committee consisting of Drs. Robert Wilson, Eric Schon, Doug Wallace and Sid Hecht led the effort to assemble sixty of the world's leading mitochondrial experts from a wide variety of fields so they could share their insights and expertise and shine a well-focused light on the kind of mitochondrial dysfunction that contributes significantly to the cause of FA and other diseases such as ALS, mitochondrial myopathies (e.g., MELAS and MERRF), Alzheimer's, Huntington's and Parkinson's, as well as diabetes and stroke. In brief presentations followed by intensive discussions, FA mitochondrial specialists were joined by counterparts in these other disorders and were able to zero in together on common ground in mitochondrial dysfunction and promising therapeutic approaches to fixing it. Many of the participants called this two-day Summit one of the finest scientific meetings they had ever attended.

To help capitalize fully on the Mitochondrial Summit, FARA and MDA announced at the conclusion of the meeting that the two organizations would offer a substantial grant for innovative research that, in the collaborative and multidisciplinary spirit of the Summit, would advance therapeutic approaches that show promise in addressing mitochondrial dysfunction in FA. FARA and MDA issued a Request for Applications and promptly received a number of excellent proposals. Each proposal was peer reviewed and scored by scientists knowledgeable in the field. The proposal selected for the award is that of Dr. Michael Koob of the University of Minnesota and Dr. Grazia Isaya of the Mayo Clinic. These two excellent investigators will receive from FARA and MDA \$100,000 per year for two years to support their effort to develop a gene-therapy approach for FA that involves delivering to FA mouse mitochondria the DNA material needed to produce frataxin protein and compensate for FA's frataxin protein deficiency.

2008 got off to a great start in mitochondrial research with Jan-



uary's two-day NIH Mitochondrial Minisymposium. That meeting brought together hundreds of experts from all over the world. FARA President Ron Bartek was invited to address the group. He emphasized that FARA recognizes that mitochondrial dysfunction is central to FA and a variety of other disorders, and proposed that the NIH add to its NIH-wide Roadmap for Medical Research a new initiative to advance mitochondrial research broadly. After much collaborative work by mitochondrial experts across multi-disease lines and between FARA and the United Mitochondrial Disease Foundation, the NIH did establish a new grant mechanism for mitochondrial research as part of the NIH Roadmap. This new funding opportunity sets aside \$25 million for 2009 to pursue progress in mitochondrial function and five other research areas.

NIH also conducted, in early October, a two-day conference on Mitochondrial Biology in Cardiovascular Health and Diseases. Approximately 500 scientists, including the Chairman of FARA's Cardiac Panel, Dr. Mark Payne and FARA's VP/Chief of Science, Dr. Giovanni Manfredi, gathered to share their insights and recent findings and to explore therapeutic approaches to mitochondrial dysfunction in heart disease, diabetes, neuro-muscular degeneration and a wide variety of other disorders.



Participants included a number of scientists who contributed significantly to the FARA-MDA Summit in Arizona, FARA grantee Dr. Michael Murphy, who discussed mitochondrial therapeutics in FA, and Dr. Gerald Shulman, who is seeing FA patient families at Yale University in his investigation of mitochondrial dysfunction in FA diabetes.

In summary, 2008 has brought a great deal of attention to the importance of mitochondrial dysfunction in human disease. FARA has helped build the momentum in this critical arena and focus much of this new attention on FA and closely related neurodegenerative disorders. The resulting increase in understanding of mitochondria will continue to instruct therapy development in FA and accelerate progress toward treatments and a cure. ■

*Pictures:*

*Top: Drs. Dave Lynch, Bronya Keats, and Rob Wilson between sessions at the 2008 Mitochondrial Summit.*

*Left: Dr. Michael Koob leads a discussion on Biodelivery at the 2008 Mitochondrial Summit.*

*Right: Jennifer Farmer, FARA Executive Director and Amale Hawi, Penwest Senior VP at the 2008 Mitochondrial Summit.*

## EDS: FARA Partner of the Year

(continued from p. 1)

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

### Key Criteria:

- Leadership and/or employees/constituents within the partner organization are directly supporting FARA's mission and demonstrate a commitment to curing FA faster than thought possible (e.g., fundraising, in-kind contributions, professional services, etc.)
- Organizational culture that values community, helping others, volunteerism
- Commitment to increasing awareness of Friedreich's ataxia or FARA

The leadership and employees of EDS, an HP company, have given generously of themselves in supporting FARA and have directly and significantly contributed to advancing our mission and bringing us closer to treatments and a cure for FA. EDS employees have been active participants in the FARA community for more than five years and provided FARA with several information technology solutions that have improved FARA programs, operations, communications and support to the scientific and patient community. In addition, these solutions have directly supported the FA scientific community in advancing clinical research in FA faster!

All of these contributions were made possible by employees volunteering their personal time and professional and technical expertise.

The programs that EDS employees have custom created for FARA and FA scientists:

- FA Natural History and Outcome Measures Database
- FARA Patient Registry
- FARA Research Portfolio Management Program
- Friedreich's Ataxia Study Group Web site

EDS employees who are skilled in communications volunteered their time to dramatically improve the FARA newsletter and create the Advocate and eAdvocate.

In 2007-2008, FARA was also the recipient of an EDS Foundation grant for \$40,000 which supported the redesign of the FARA Web site, necessary IT infrastructure and launch of FARA's education and awareness initiative.

FARA Executive Director, Jennifer Farmer, has been fortunate to work with all of the EDS volunteers on each of these projects. She is amazed by the compassion and generosity of each person she has encountered. Most of the people do not have a personal connection to FA but they clearly have a goodness and desire to help others.

On behalf of FA scientists and families everywhere, FARA would like to express its deep appreciation to FARA's Partner of the Year for 2008, EDS. ■

### EDS Volunteers

#### Ataxia Scales (2002-2004)

Marianne Wilcox, Margaret Ferrarone, Leo Bellew, Chris Naylor, Bill Hartnett, Rich Dusse, Alice Bearce, Christine Ward, Sherri Stone, Linda Guest, Marty Ohman, Doug Luffman, Ali Sleiman, Barbara Tate, Terry Vanfleet, Yubin Ye, John Jones, Glenn Lawrence, Jeff Hartwell, Laura Backhaus, Sheryl Barefoot, Mike Best, Gregory Cebollero, Keith Crotty, David James, Scott Keller, Tim McElheny, Jill Werner, Darla Messenlehner, Bob Skyles, Ann Titus, Terry Trusik, Steve Selak, Rip Henry and Barbara Kassnoff

#### Registry (2005 – Present)

Michelle Whalen, John Manos, Don Pecor, Darren Bielby, Julian Pisoni, Dennis Skrtic, Michael Vonwald, Rod Clingaman, Brad Morse and Steve Baiera

#### RPMP (2006 – 2008)

Marty Ohman, Christine Ward, Allen Chamberlain, Chris Naylor, Terry Van Fleet, Lisa Hupf, Jill Church, Jill Werner, Doug Luffman, Rich Dusse, Rick Huntoon, Lew Tomeno and Maribel Santiago-Ostolaza

#### Communications (2002 – Present)

Mary Beth Kosmicki (communications advisor), Meg Giaconia (Advocate/eAdvocate Publisher), and Amy Goodno (2005)

#### Friedreich's Ataxia Study Group (2006-2007)

Curt Moczarski, Brent Hinline, Larry Bese and Steve Baiera

#### FARA Board Members (2005 – Present)

John Cubbin and Bill Hartnett

#### FARA CIO (2005-Present)

Marianne Wilcox

EDS's countless volunteer hours are inspired by the Ferrarone Family. Margaret Ferrarone is an employee at EDS who has two daughters with FA, Sara and Laura. The EDS employees have united on behalf of Margaret's girls to provide FARA with the IT Infrastructure necessary to facilitate FA research.

## Featured Fundraiser

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



### Nelda Van Schoick

By Marilyn Downing

When asked about her motivation for fundraising, Nelda Van Schoick stated, "I do this to honor my girls, Becca and Robbi. They know they are a part of the progress by helping with the fundraisers. They think in terms of it helping other people...the younger ones coming up behind them."

Along with helping her two lovely and intelligent girls with FA achieve their full potential, Nelda has also made fundraising a priority in her life. She began fundraising in 2001 when her friends wanted to plan a going away party before the family moved from Texas to Georgia. With the recent initiation of Friedreich's Ataxia Awareness Day, Nelda began thinking outside the box and instead asked her friends to help with a fundraiser for FA research.

Once in Georgia, Nelda and friends held neighborhood walks to raise money. Nelda thought about growing her fundraisers, taking into consideration the interests of her audience. She knew people would enjoy a party and so they sent out invitations to their Backyard Bash, set a suggested donation, and held a small silent auction. Though her event was successful, Nelda felt that her future events might benefit from the support of larger donors if she appealed to their interests. The Van Schoick family is fortunate to have some work connections with some "very good and very generous people." Knowing the interests of those individuals, she and her husband Bob began organizing a golf tournament, held the same day as their infamous "Backyard Bash." The golfers came ready to party right off the course.

This year there were two Van Schoick events, a golf tournament and Benefit Bash in Bogart, GA and Lewisville, TX. Over the years the Van Schoick family has raised over \$200,000 for FA research. Any amount is helpful. As Nelda says, "If 100 families each raised \$5,000 a piece—think how much money that is!" Nelda's ambition, expertise and common sense are a blessing for all in the FA community.

#### Van Schoick Words of Wisdom:

- Start small and build. This is so much less intimidating and gives you time to find out what works and what doesn't. Eliminate what doesn't work and try something new.
- Fundraising is highly individualized according to a family's time, abilities, and motivation. Find your base of support and figure out what they like to spend their money on.
- Be a good friend to other people by helping them

with their fundraisers. Generosity begets generosity.

- Start planning months ahead. Start with a good committee (honest and trustworthy) and learn their individual strengths for specific tasks (e.g. auction, invitations, Web site design, running the last minute errands).
- Plan an activity that interests all members of your family. If your child doesn't want to be in the lime-light, consider doing a letter writing campaign.
- Don't hesitate to ask. You will sometimes be surprised at who will be your major supporters.
- Start and maintain a Web site for your own event. Name the Web site (e.g. [www.benefitbash.org](http://www.benefitbash.org) and use it year after year.) Keep it updated, include photos, and tell people where their money went.
- People need to be able to give even if they don't attend the event. This can be handled through a link on your event Web site or through the FARA Web site.
- Events have a "lifespan" so be open to change and be sure people continue to have a good time. ■

*More FARA Fundraising on p. 13*

### In Memory. . .

From January 2008 to October 2008, FARA has received \$12,500 in memory of the following individuals:

John, Andresen, Spiros Angelopolous, Thomas Barnett, Ann Browne, Carmela Caruso, Corrin Cooper, Jan DeSaw, Bruce Ebert, Paul Flippo, Beulah Gonyer, Grace Hopp, Havina Huellen, Grace Kelly, David Lewis, Frederick Liuzzo, William Lyons, Kevin Maher, William Massey, Ellie Mae McManus, Assunta "Sue" Mullaney, Betsy Neyland, Beverly O'Connell, Larry O'Connell, Leo Pryor, James David Rudell, Maureen Smith, Dr. William Stifter, Ed Swafford, Dalton Wallace, Barbara Western, Anita Woody.

Thank you to all who chose to remember a loved one with a donation to FARA.

To request envelopes to be used for memorials, please contact FARA at [info@cureFA.org](mailto:info@cureFA.org)

## FARA Officers & Directors

*FARA would like to acknowledge and extend a special thank you to these very generous donors.\**

### **\$10,000-\$25,000**

- Apopharma
- Andrew & Melora Balson Family Fund
- Anonymous Donor in Support of Sally Braid's Ironman
- Chesapeake Bay Area Charities
- DST Systems
- MedPharmex Animal Health
- Miller Brewing Company
- Performance Awards Center
- Performance Food Group
- Nancy Schneid
- Silicon Valley Community Foundation

### **\$45,000-\$99,000**

- Mark Aaron
- Bonefish Grill
- Venrock Partners

### **\$100,000 and Above**

- Avery Family Foundation
- Standard Meat Company

*\*January 2008 to October 2008*

**Ronald J. Bartek**, President/Director/Co-Founder  
Retired U.S. Government Official; Business Consultant

**Thomas A. DeCotiis**, Chairman of the Board  
Founder and Chief Executive Officer, CorVirtus

**Marilyn E. Downing**, Secretary/ Director  
Teacher/ Diagnostician, Special Education

**Terrence M. Downing**, Treasurer/ Director  
Certified Financial Planner and Certified Public Accountant

**Paul Avery**, Director  
Chief Operating Officer, OSI Restaurant Partners, LLC

**John Cubbin**, Director  
Vice President, EDS, an HP company

**Nicholas A. Johnson**, Director  
Associate & Senior Mechanical Engineer, Bard, Rao + Athanas  
Consulting Engineers, LLC

**Laura Kalick**, Director  
Nonprofit Tax Director, BDO Seidman, LLP

**Paul Marcotte**, Director  
Attorney & Communications Consultant

**Peter Pitts**, Director  
President, Center for Medicine in the Public Interest  
Senior Vice President, Director for Global Health Affairs for  
Manning Selva & Lee

**Edward Ramsey**, Director  
Co-Owner and Vice President, Taylor Ramsey Corporation  
Head of International Trade Division

**Nancy Schneid**, Director  
Brand Consultant, OSI Restaurant Partners, LLC

**Dr. Earl Giller**, Scientific Director  
Consultant, Global CNS Pharmacology Consulting, LLC

**Dr. Bronya J. B. Keats**, Scientific Director  
Geneticist, Louisiana State University

### **FARA Advisors:**

**Mary Beth Kosmicki**, Communication Advisor  
Worldwide Marketing and Communications Leader,  
EDS, an HP company, Symantec

**Mary Caruso**, Development Advisor  
Small Business Owner

**William Hartnett**, IT & Development Advisor  
Program Manager, EDS, an HP company

**William Krutzer**, Development Advisor  
Louisiana State Commissions

**Sandy Lane**, Development Advisor  
Small Business Owner

**Samantha Litke**, Development Advisor

**Marianne Wilcox**, IT Advisor  
Enterprise Architect, EDS, an HP company

FARA would like to thank the following families for inspiring generous giving in their communities by working with family, friends, neighbors, local schools, sports groups, and small businesses.

The Ashman Family  
The Bellnier & Brown Families  
The Bianchi Family  
The Dalton Family  
The Gambill Family  
The Gill Family  
The Hanson Family  
The Kittel Family  
The Krause Family  
The Lane Family  
The Lawson Family  
The Ostby Family  
The Pepitone Family  
The Price & Austin Families  
The Wojenski & Franz Families  
The Wolfson Family



## FARA Fundraising

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

### "Let Go. Give Back." Outback Steakhouse Fundraising for FA Research

By Felicia DeRosa

In July 2008, Outback Steakhouse restaurants started hosting luncheons to raise funds for FA research. Many Outback restaurants have generously donated the event invitations, their location, their food, and their services so that all the proceeds from luncheon ticket sales go to FARA to support FA research. Keeping with FARA's tradition of local, grassroots fundraising, numerous Outback restaurants and FA families teamed up to host luncheons across the country. FA families have given their time to help promote the events and bring in attendees. Raising critical awareness, they also spoke to guests about life with FA and the research that FARA supports. A special thank-you to the Luebke Family and the Konanz Family for partnering with Outback in Ohio and California respectively to orchestrate the multi-restaurant initiative in those areas. Thank-you also to all of the Outback staff who not only warmly welcomed guests and generously gave their time to host the luncheons, but also donated their tips to further boost the fundraising totals for FA research.

In addition to the luncheon model, a number of the Outback restaurants and FA families were creative with their fundraising by initiating auctions, selling FARA/ Outback pins (Waynesboro, VA), organizing successful golf tournaments (Altoona, PA; Northern Ohio; Richmond, VA), creating FARA sponsorship hearts (Waldorf, MD), hosting a softball tournament (South Texas), and hosting a Clay Shoot Tournament (Chesapeake Bay Area Maryland). As of early October 2008, the Outback locations and FA families listed below have raised an estimated \$150,000 for FARA in the short time since this project was initiated. FARA is sincerely grateful to all of the Outback teams and FA families who have given their time and talent to this initiative.

#### CALIFORNIA

The Rohnert Park Team

#### GEORGIA

The Buckhead Team; The Valdosta Team

#### KENTUCKY

The Crescent Springs Team

#### LOUISIANA

The Lafayette Team

#### MARYLAND

The California Team; The Chesapeake Bay Area Team; The Hyattsville Team; The LaPlata; The Laurel Team; The Oxon Hill Team; The Waldorf Team

#### NEW JERSEY

The Rochelle Park Team; The Ledgewood Park Team

#### NEW YORK

The Bayside Team; The Clifton Park Team; The Staten Island Team; The White Plains Team; The Yonkers Team

#### OHIO

The Centerville Team; The Cincinnati/ Colerain Team; The Cincinnati/ 5 Mile-Beechmont Team; The Cincinnati/ Kenwood Team; The Cincinnati/ Springdale Team; The Cincinnati/ Western Hills Team; The Mason Team; The Northern Ohio Team

#### PENNSYLVANIA

The Altoona Team; The Bensalem Team; The Lancaster Team; The Monroeville Team; The Moon Team; The North Hills Team; The South Hills Team; The Washington Team

#### TEXAS

The Corpus Christi Team; The Fort Worth & Burleson Teams; The Grapevine, Irving, & Hurst Teams; The Westheimer/Houston Team; The Lewisville, Denton, & Addison Teams; The Mesquite & Dallas/Greenville Teams; The Plano, Garland, and Frisco Teams; The South Arlington & DeSoto Teams; The Laredo Team; The San Marcos Team; The McAllen Team; The South Texas Teams

#### VIRGINIA

The Alexandria Team; The Arlington Team; The Newport News Team; The Richmond Team; The Vienna Team; The Waynesboro Team

#### FA HOST FAMILIES

The Bartek Family; The Cernosek Family; The Cusick Family; The Crowley Family; The Ferguson Family; The Gentry Family; The Hammer Family; The Henry Family; The Jacks Family; The Jimenez Family; The Konanz Family; The Krivinko Family; The Luebke Family; The Mailloux Family; The McDonnell Family; The Miller Family; The Richard Family; The Simpson Family; The Aaron Smith Family; The Mackenzie Smith Family; The Sally Smith Family; The Weigand Family; The Welch Family; The Weyent Family; The Zies Family



#### Pictures:

Top: Tammy Luebke, Kent Little (Outback), and Pamela Rasey at an Outback fundraising luncheon in Ohio.

Bottom: Brianne Konanz and the Rohnert Park, CA Outback staff.

More FARA Fundraising on p. 14

## FARA Fundraising (continued from p. 13)

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

By Felicia DeRosa and Marilyn Downing

In 2008, FARA's grassroots fundraising program grew in both the size and number of events. Veteran fundraisers continued to impressively grow the fundraising capacity of their events and many new families joined the fundraising team hosting very successful first time outings.

As illustrated below, FA families from across the country organized a diverse range of events, volunteering countless hours and rallying the support of their communities. 2008 included several sporting fundraisers such as walk-a-thons, golf tournaments, bike tours, a kickball tournament, marathon runs, and a triathlon. One fundraiser even completed an ironman competition in the pouring rain. This year's dinner dances and parties had wonderfully creative themes and touching messages such as a Hawaiian luau complete with leis and a handcrafted tiki bar (FA-ITH), good summertime fun at backyard BBQ parties, a balloon theme for the cleverly named Up, Up, and Away with FA, the message of kindness at the Find A Cure Dinner where event organizers gave each guest a small pewter heart, and the inspiring philosophy of Dream, Hope, Believe at the Crab Feed event.

At the start of October 2008, FARA's grassroots fundraising net proceeds funded more than half of FARA's grant program to date. On behalf of the rest of the FA community, thank you grassroots fundraising team. You inspire us with your energy and dedication to FARA's mission. It is the momentum propelling the research forward.

2008 EVENTS	EVENT PLANNERS	LOCATION	IN HONOR OF	AMOUNT RAISED
<b>NORTHEAST</b>				
Boston Marathon	John Luth and Randy Mann	Boston, MA	Joel Kleine	\$3,112
Buffalo Wild Wings Restaurant Event	Ashlea Weigand	Robinson, PA	Ashlea Weigand	\$1,363
The Bullpen Open Golf Tournament	Jason Krogmann	Saratoga Springs, NY	Dylan McDonnell	TBA
*FA Fighters 5K Walk	Danielle Minarchik	Philipsburg, PA	Alex Wilkinson	\$20,735
Find A Cure Dinner/ Auction	Mary Caruso	Branford, CT	Sam and Alex Bode	\$60,000
*Five Borough Bike Tour	Mike Nelin & Kevin Mulvaney	New York, NY	Brian Mulvaney	\$9,404
Fuzzy Buzzy Golf Tournament	Mary Ann O'Neil & Uncle Paul Staniech	Windham, NH	Erin O'Neil	\$13,000
*Jonestown Men's Choral Concert	Dennis Wood	Jonestown, PA	Dennis Wood	TBA
*Lake Placid Iron Man	Sally Braid	Lake Placid, NY	Donovan Simpson	\$26,505
*Music Festival	Dylan and Dave McDonnell	Glens Falls, NY	Dylan McDonnell	\$4,555
*Osceola Mills Elementary School Walk & Fundraising	Osceola Mills Elementary School	Philipsburg, PA	Alex Wilkinson	\$9,981
Olsen Golf Tournament	Dan Olsen	Monroe, NJ	Dan Olsen	TBA
The Voyces Concert	Brian Wurschum	New York, NY	John Cernosek	\$900
Welsh Backyard Bash II	Peter and Francine Welsh	Harrisburg, PA	Brendan Welsh	\$21,662
Westchester Triathlon – Team Donovan	Norm & Debra Simpson Jennifer Sinnott	Rye, NY	Donovan Simpson	\$26,700

\*-Indicates first time fundraiser for Friedreich's Ataxia.

TBA—The amounts raised for these events were not finalized at the time of printing

**2008 Fundraising Chart continues on p. 15**

2008 EVENTS	EVENT PLANNERS	LOCATION	IN HONOR OF	AMOUNT RAISED
<b>SOUTHEAST</b>				
BRO Golf Tournament	Armando Martinez	Washington, DC	"Rosie" Rosencranz	\$13,000
Cernosek Kickball Tournament	Laura Cernosek	Arlington, Va	John Cernosek	\$4,260
Golf Tournament and Benefit Bash	Nelda Van Schoick	Bogart, GA	Robbi and Becca Van Schoick	\$90,242
Gymnastics "Leaping for a Cure" Casino night	Karla Wooten	Gainesville, FL	Josh Wooten	\$8,320
<b>MIDWEST</b>				
4 <sup>th</sup> Annual Race for a Cure	Tammy Luebbe	Cincinnati, OH	Evan Luebbe	\$24,200
*Flying Pig Marathon	Eileen Cameron	Cincinnati, OH	Evan Luebbe	\$1,700
*Hole Out for a Cure- Rick Peters Charity Golf Tournament	Rick Peters	Peoria, IL	Rick and Todd Peters	\$26,700
<b>NORTHWEST</b>				
*Dream, Hope, Believe- Crab Feed	Vicki Hartigan	Rancho Cordova, CA	Ashley Hartigan	\$12,440
	Kyle Bryant & Sean Baumstark	Sacramento to Las Vegas	Sean Baumstark, Beth Bax, Sam Bridgman, Kyle Bryant, David Henry, Linda Johnson	\$142,000
Ride Ataxia II Cycling				
*Ride Ataxia III Party	Angela Lacativo Greene	Martinez, CA	Kyle Bryant	\$7,000
*Up, Up, and Away with FA Dinner/Auction	Debbie Austin	Spokane, WA	Jessica Austin	\$40,000
<b>SOUTHWEST</b>				
FA-ith (FA in the Heart) Dinner dance	Rupel Family	Sunnyvale, CA	Matt Rupel	\$80,310
*Lone Star Benefit Bash/Castle Hills Golf Tournament	Vince Palasota Jerry Russell	Lewisville, TX	Robbi and Becca Van Schoick	\$42,660
*San Francisco Bay Area Walk	Trent Gaylord	San Francisco, CA	Brianne Konanz	\$1,515
Race for Results	Carrie Laird	Bakersfield, CA	Jerod Laird	\$7,950
Stephanie's Hope Holiday Boutique	Sharon Magness	Valencia, CA	Stephanie Magness	TBA

\*-Indicates first time fundraiser for Friedreich's Ataxia.

TBA—The amounts raised for these events were not finalized at the time of printing

## Upcoming Events - First Quarter 2009

Betsy & Emily's Fight to End FA- December 13, 2008  
Fort Stockton, TX

[Ruth\\_neyland@hotmail.com](mailto:Ruth_neyland@hotmail.com)

Stars for a Night - A Glimmer of Hope- January 24, 2009

Rochester, NY

[DACBrown@rochester.rr.com](mailto:DACBrown@rochester.rr.com)

Fox Chapel Area High School Alumni Orchestra  
Concert- January 8, 2009

Pittsburgh, PA

[Mairi\\_Thompson@fcasd.edu](mailto:Mairi_Thompson@fcasd.edu)

Ride Ataxia III- March 16-19, 2009

Portland, OR to Seattle, WA

[www.rideataxia.org](http://www.rideataxia.org)

Third Annual Race 4 Results- March 28, 2009

Bakersfield, Ca

[Keyedup@bak.rr.com](mailto:Keyedup@bak.rr.com)

St. John's UUC of Jonestown FA Dinner/ Walk- April 25 & 26, 2009

Jonestown, PA

[Dwood02@comcast.net](mailto:Dwood02@comcast.net)

the insulin-producing cells of the pancreas. When a person dies from the complications of the disease, a rapid autopsy will allow researchers to harvest important organs for detailed study. The goal is to learn as much as possible about FA and apply new knowledge to diagnosis, treatment, and care of other patients with the disease.

For information and formal enrollment in a research program, contact:

Dr. Arnulf H. Koeppen

VA Medical Center

113 Holland Ave

Albany, N.Y. 12208

Tel. 518-626-6391; 518-626-6377

FAX 518-626-6369

e-mail: [arnulf.koeppen@va.gov](mailto:arnulf.koeppen@va.gov)

## Parent Perspective

Mary-Lisa is a parent who has gone through this process. She offers her compassion and wisdom as she helps organize the donation. Studies of her son's organs have played a key role in understanding the effects of the disease on heart and other organs.

For additional information for families on donations and help making the arrangements, contact:

Mary-Lisa Orth

FARA Tissue and Organ Donation Liaison

10000 N Feldman Rd

Tucson, AZ 85653

Tel. 520-616-9883

Cell 520-403-0577

e-mail: [Rocketmom@att.net](mailto:Rocketmom@att.net)

FARA e-mail: [Mary-Lisa.Orth@curefa.org](mailto:Mary-Lisa.Orth@curefa.org)

The FA research community is optimistic about advances in our understanding of the disease and new treatments. Many of the recent breakthroughs are based on research that has only been possible because FA families have donated tissues. ■

## Autopsy and Tissue Donation Program

FARA supports a research program at the VA Medical Center in Albany, New York. The principal investigator, Dr. Arnulf Koeppen, is a neurologist and neuropathologist and has made significant contributions to our understanding of FA and other ataxias. He has provided insight about severity of illness and cause of death to many FA families who lost a loved son, daughter, brother, sister, wife, or husband. Dr. Koeppen collaborates with many FA researchers all over the world. He shares his experience with other investigators and makes available valuable FA tissues for ongoing and future research.

## Tissue donation

Patients with FA and their family members are invited to inquire about autopsy and tissue donation for research. Friedreich's ataxia is a very complex disease and affects spinal cord, nerves, brain, heart, bone, and

## FARA Launches New Web site

Visit [www.cureFA.org](http://www.cureFA.org) for updated information on FARA, FA Research & Clinical Program, Patient & Family Resources, and Ways to Support FA research.





*Brenda, Katie, Matt, and Bart Rupel at their Hawaiian themed FA-ITH fundraiser.*



*Paul Konanz, half of FA-ITH's book-keeping dynamic duo.*



*Jerry Russell and Vince Palasota, generous supporters of the Van Schoick events, along with Ron Bartek, Becca and Robbi Van Schoick.*



*Ashlea Weigand and a new friend at an Outback fundraising luncheon in Pittsburgh.*

*The Hartigan Family overwhelmed by the generosity of their community at their fundraising event, Dream Hope, Believe- Crab Feed.*



**The FARA Advocate is brought to you by:**

**Editors: Ron Bartek, Felicia DeRosa, Marilyn Downing, Jennifer Farmer, Mary Beth Kosmicki, Paul Marcotte**

**Advisor: Bill Hartnett**

**Design/Layout: Meg Giaconia**





Marilyn Downing, Alec Brown, and Karen Brown at Alec's Play for FA school game night.



The registration desk getting ready for the golfers at the Fuzzy Buzzy golf tournament.



Tom Trovinger and Erin O'Neill at Erin's fundraiser, The Fuzzy Buzzy Golf Tournament.



Team Donovan gathers for a pre-race picture on the beach at Rye Playland, the site of the Westchester Triathlon.





*The Northern Ohio Outback Team with the FARA banner at their fundraising golf tournament.*



*Ron Bartek, Sam Bode, Alex Bode, and Mary Caruso enjoy a slideshow at the Bode/Caruso's fundraiser, Find A Cure dinner/auction.*



*Outbackers, Christine, Jen and Keslie staff the registration desk at their fundraiser, Swinging for A Cure in Altoona, PA.*





Marilyn Downing and Dr. David Lynch at the Bode/Caruso's fundraiser, Find A Cure dinner/auction.



Brendan, Francine, Peter, and Eilish Welsh at their fundraiser, Backyard Bash II.



Eilish and friends staffing the registration/raffle ticket table at the Welsh's Backyard Bash II.



Runners sprint in honor of Jerrod Laird at the Second Annual 10K for FA in Bakersfield, CA.



Aaron Smith and the Lafayette, LA Outback staff.



Jim (aka Jimbo) and Karol Price and Dick & Deb Austin, event coordinators for the Up, Up and Away with FA event.





*The Mulvaney and Nelin clans at the Five Borough Bike Tour, NYC, May 2008*



*John Luth and Randy Mann of St. Albert (Alberta, Canada) run the Boston Marathon in May in honor of their friend with FA, Joel Kleine.*



*Mary and Andrew Muir, two very important players in the Peters' family's Hole Out for a Cure.*



*Rick, Jean and Gordon Peters at their Hole Out for A Cure in Peoria, IL.*



*Participants in the San Francisco Bay Area Walk.*





*Evan Luebbe at his family's fundraiser, The Fourth Annual Race for the Cure.*



*Kyle Bryant with the hosts of the Backyard party that kicked off fundraising for Ride Ataxia III, his cousin Angela Lacativo Greene and her husband Ricky Greene.*



*Creative "spare change piggy banks" to collect money for research in honor of the Wolfson family (thanks to Stacia McCann, art instructor at Garden Lakes School in Avondale, AZ).*



*Kyle Bryant with Outbacker Patrick Kruk (second from right) and his team at a Backyard party fundraiser for Ride Ataxia III. Patrick and his Outback team generously donated the food and service to support this event.*



*A member of Team Donovan races to the finish at the Westchester Triathlon.*





Alex Wilkinson leads his schoolmates from Osceola Mills Elementary School in a walk to raise awareness and funds for Friedreich's Ataxia.



This beautiful quilt will be raffled by the Bedford Friendship Quilt Guild (NH). Email [hollis.hope@comcast.net](mailto:hollis.hope@comcast.net) for information.



The FA Fighters walk-a-thon event had something for everyone. While walkers braved scattered rain and listened to tunes from a live DJ, the kids found fun under the tent with games, face painting, and baked goods.



At the end of the FA Fighters Walk-a-Thon, Alex Wilkinson takes a break from cruising on his new wheels for a picture with Mom- Danielle, Little Sister- Bella, and Friend- Randi.



Ashley Hartigan (right) and her friends gather around the Balloon Popalooza table at the Hartigan's fundraising event, Dream, Hope, Believe- Crab Feed.

## Contact Us



Friedreich's Ataxia Research Alliance  
P.O. Box 1537  
Springfield, VA 22151

Phone: (484) 875 3015

E-mail:  
[info@cureFA.org](mailto:info@cureFA.org)

Web Site:  
<http://www.cureFA.org>

**Friedreich's Ataxia Research Alliance**

If you would like to be removed from FARA's mailing list, please e-mail [info@cureFA.org](mailto:info@cureFA.org).