The Advocate

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FARA Friedreich's Ataxia Research Alliance

Issue #11 Winter 2010-2011

From the Executive Director

Shaping FARA for Success

By Jennifer Farmer

FARA has had a challenging year, which has only made us stronger, wiser and more determined. We are all too often reminded that FA is a devastating disease. In January, Keith Michael Andrus, whose diagnosis of FA inspired the foundation of FARA, was taken from us. Keith's death and the losses that every individual and family with FA face are what sets the urgent pace of FARA's activity.

As the Executive Director, one of my primary responsibilities is to ensure that FARA's operations and programs execute our strategic initiatives and support the mission: treatment and a cure for FA. FARA's 2010 strategic initiatives are reported on throughout this issue of *The Advocate,* but below is a snapshot of the infrastructure enhancements to support this overall strategy.

Advancing Research – FARA is the leader in funding FA research through our grant program. FARA's scientific advisors provide critical direction to this program and to our strategic research initiatives. FARA is taking a proactive approach by soliciting grants that advance lead candidates, technologies, knowledge, or resources and translate discoveries made in the laboratory to treatments. In addition, FARA has established a Scientific Review Committee to oversee the grant review process and follow up with FARA-funded scientists to carefully steward the funds going to research.

Fundraising/Development – With investment over the past two years to build enhanced infrastructure for event-based fundraising, we have had a successful year with the 2nd FARA Energy Ball, launch of national Ride Ataxia events, and Team FARA.

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FARA's Research Grant Program

By Jennifer Farmer

FARA's primary force toward advancing research is our grant program. FARA receives grant proposals from researchers all over the world – 24/7/365. We don't have deadlines that require researchers to wait six months or a year before submitting a proposal. Recently, FARA began to proactively solicit for research proposals to fill gaps in our research portfolio (e.g. cardiac research) or to address specific needs in the research community (e.g., access, characterization, and optimization of FA mouse models). Each proposal is detailed, containing a hypothesis, specific aims, preliminary data, research plan, credentials of the scientific team, and a budget. Each grant proposal goes through both an internal and external scientific review process prior to FARA making a funding decision. This review process is critical to evaluating the scientific validity, significance, and research plan being proposed.

In 2010, FARA has received more than 35 new grant proposals and 15 requests for continuation of funding on multi-year proposals, resulting in more than 30 funded research grants exceeding 2.5 million dollars. These grants covered a wide range of research. Some of the grants address the need to further understand the basic science, such as the role of frataxin and cellular pathways implicated in FA. Several projects focus on developing improved neuronal and cardiac cellular models of FA that will allow researchers to better understand what is happening to these cells as part

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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. 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 Letter

Dear Friends,

We all recognize what a difficult year 2010 has been for the FA family. We have lost some truly fine, deeply loved young people to FA this year. As has become a hallmark of the FA family, though, we are all grieving together for those we have lost, but at the same time, we are celebrating their lives and the mission they inspired us to take on together. We are also recommitting ourselves—partly in their legacy—to accomplishing that mission together. More than ever, failure is not an option.

As 2010 enters its final weeks, I would like to share with you the encouraging and invigorating global forces that are coming together to help us meet the challenges we face and accomplish our mission of treatments and a cure for FA. FARA and the FA community have worked hard over the last dozen years to build the worldwide partnerships we knew would be required to get the job done. Now, just when we need it most, the power of those partnerships is fully coming to bear and the growing momentum behind the global effort to treat and cure FA is building into the irresistible force that will conquer what once seemed to be the immovable object of FA. The force is with us!

What are the key elements of this powerful force? 1) Patient advocacy organizations around the world are increasing their collaborations with FARA in support of the research. Organizations in Australia/New Zealand, Europe and North America are co-funding grants with FARA and many of them will join us at the 4th International Scientific Conference on FA next May in France. 2) The pharmaceutical industry—even larger companies—are becoming increasingly interested in rare diseases and many of them see FA as an excellent focus for them because the FA family has supported and matured much of the "discovery science" and is providing a great deal in the way of necessary drug development tools and clinical infrastructure. We are in early stage discussions with several new companies interested in joining the effort and we are confident that some of these partners will be added to our research pipeline in 2011. 3) Government agencies are stepping up their support of FA research. The U.S. National Institutes of Health has funded numerous FA research grants, supported each of our international scientific conferences, paid for and conducted two phases of an FA clinical trial, helped fund the preclinical drug development of one of our promising drug candidates now in clinical trial, and recently accepted the second FA project into its ultra high-throughput screening program used to screen, in just a few days, 350,000 compounds for effectiveness in FA. The U.S. Food and Drug Administration is becoming more and more interested in FA and in helping us advance FA drug candidates. The European Union has awarded a grant of about $10 million to a European consortium of FA researchers. 4) The number of scientists devoting their careers to FA research continues to grow. Only 80 scientists participated in the 1st International Scientific Conference in FA but we will have to turn away a number of them to keep the participants in the 4th Conference next May to a manageable 200. 5) A growing number of FA patients are participating in clinical trials around the world. With the number of clinical trials under way now and soon to be launched, it is increasingly important that all FA patients enroll in the FARA patient registry (www.curefa.org/registry) and participate in the trials. 6) An increasing number of FA families, friends and supporters are stepping up to conduct, participate in, and donate to the FARA fundraising activities absolutely essential to our unflagging support of the research. Thank you very much. This issue of The Advocate *represents FARA's final appeal of 2010 for your financial support. If you have not yet had the opportunity to make a donation this year to FARA's research efforts or if you might be able to make another before the end of the tax year, please consider doing so now.*

I hope you can see and feel the powerful force of partnership that is steadily mounting in the concerted effort to treat and cure FA. Yes, the FA family has known severe challenges, and there will be more ahead. But, with each of us playing our own important role, together, we must and will prevail.

Warmest regards,
Ron

Acting alone, there is very little any of us can accomplish.
Acting together, there is very little we will NOT accomplish.

Communications/Raising Awareness – To make more significant strides in raising awareness about Friedreich's Ataxia and FARA, and to enhance the FA Community presence through events in multiple media markets, FARA decided to work with a public relations firm, The Star Group. The Star Group is focusing its work in three program areas; general awareness through traditional and social media; growing Ride Ataxia through event participation and sponsorship; and outreach to the greater pharmaceutical industry to leverage the recently recognized value of drug development for rare diseases and generate new interest in Friedreich's ataxia research.

During the past several years FARA has experienced significant growth as an organization in direct response to advancing our research pipeline. The FARA board of directors and staff constantly evaluate this growth and make adjustments along the way to keep the organization lean and efficient. It is frequently said internally that we are not about growing an organization to grow an organization, we are about reaching our goals faster than thought possible through our core values: wisdom, quality, spirit, caring, integrity, and urgency.

To increase transparency we prepared our first annual report release earlier this year. This was a detailed report of 2009 financials and programs. We will do the same in early 2011, preparing a 2010 annual report with audited financial statements and reports on our key research, awareness and fundraising initiatives. FARA annual reports and tax filings (990) are available on our website. We have continued to closely monitor our expenses and anticipate that fundraising and operational expenses will still be below 20% even with the vast majority of our income derived from event-based fundraising. We have experienced a decline in individual gifts; as have many non-profits during this difficult economic climate; however, our event-based fundraising has not only continued to sustain our programs but has grown and allowed us to fund more research grants than in 2009.

Twice this year I witnessed a finish-line crossing that brought to me a vision of the future where we collectively cross the finish line with treatments and a cure for FA: once, at the end of Race Across America – witnessing the culmination of extreme effort, teamwork, and determination as Team FARA crossed the finish line with two individuals with FA (Kyle and Sean) leading the way – and again, demonstrated by Sam Bode at Ride Ataxia Philadelphia. I have known Sam for more than 12 years, since she was a little girl recently diagnosed with FA. She has always been a determined fighter in spirit with a winning smile. Sam completed the 10-mile challenging ride on a recently acquired hand-cycle. Waiting at the finish line, I watched Sam turn the crank, each turn purposeful and monumental. I keep that vision of Sam firmly in mind, so symbolic of the ground we have covered and what remains ahead of us. Kyle best described FARA's staff, directors, and all our volunteers and supporters in his recent video talking about the Race Across America experience – we are driven by purpose and we just need to keep turning the crank. We must remain strong, focused, determined, and purposeful. ■

FARA's Research Grant Program

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of the disease, but also to provide efficient and effective systems for testing novel therapeutic approaches. Drug discovery and pre-clinical development of therapeutics are the focus of several grants. FARA has funded clinical research such as biomarker studies, a hearing loss study, clinical trials, and provided funding for the ongoing natural history study, cardiac and mortality study, biorepository, and functional measure development through grants to the Collaborative Clinical Research Network in FA. A full list of funded grants is on the FARA website: **www.curefa.org/grants-awarded.html**. FARA's grant management system has a partial listing of the grants with summaries of the projects online at: **www.curefa.org/RPMP/public/pggrantlist.aspx**.

In order to ensure that these research grant funds are optimized and carefully stewarded, FARA has instituted additional oversight and review practices. The FARA Scientific Review Committee (SRC) oversees the entire grant program — reviewing all grant proposals and progress reports, managing the external scientific reviews, and making funding recommendations to the Board of Directors. SRC members are in regular contact with all FARA grantees to check on progress and results, and facilitate collaborations. FARA enters into a formal grant agreement with any grantee/institution to ensure mutual understanding of the terms and limits of the award.

FARA continues to partner in research funding with other FA advocacy groups and research associations funding FA science: American Heart Association, Ataxia UK, FARA-Australasia, Friedreich's Ataxia Society of Ireland, GoFAR, and the Muscular Dystrophy Association. Our partnerships do not focus solely on funding but also on ensuring that our grant programs are complementary and not overlapping or duplicative. We also work together to facilitate scientific meetings and conferences, such as the 4th International Friedreich's Ataxia Scientific Conference to be held in Strasbourg, France, May 5–7, 2011.

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As a champion of public-private partnership to advance research, FARA is also a partner in funding some of the pre-clinical/drug development research that must be done to advance a drug from the laboratory to human studies.

Recently Awarded Industry Grants

Study of EPI-743 on visual function in patients with Friedreich's ataxia

Principal Investigator: Dr. Guy Miller, Edison Pharmaceuticals

Edison's approach centers on the consistent observation that CoQ10 registers a "signal" in a variety of diseases. This has been observed in diseases of interest to Edison — inherited mitochondrial diseases — but has also been observed in neurodegenerative and metabolic disease, now sometimes referred to as "diseases of aging." The Edison team has asked two questions: (1) If the CoQ10 effect is real, can a better CoQ10 be devised? (2) If a better CoQ10 can be devised in the lab, can it be studied in a rational phase 2B study? By rational, we mean against biomarker endpoints in a ~ 40 person study where dose finding and indicators of therapeutic effect are (or can be) observed, as opposed to effectively a cost- and time-intensive phase 3 study using standard clinical metrics of improvement on >100 subjects. Today, EPI-743 represents the most advanced clinical candidate in Edison's portfolio.

Identification of backup compound for HDAC Inhibitor RG2833 to treat Friedreich's ataxia

Principal Investigator: Dr. James Rusche, RepliGen Corporation

This application concerns the development of a novel class of histone deacetylase (HDAC) inhibitors as therapeutics for the neurodegenerative disease Friedreich's ataxia (FRDA), through a collaborative effort between the Gottesfeld laboratory at The Scripps Research Institute (TSRI) and Repligen Corporation. Unlike many triplet-repeat diseases, expanded GAA triplets in FXN are in an intron and do not alter the amino acid sequence of frataxin protein; thus, gene activation would be of therapeutic benefit. Based on the hypothesis that the acetylation state of the core histones might be responsible for silencing expanded FXN alleles, we identified HDAC inhibitors as potent activators of FXN transcription in cell culture. These HDAC inhibitors cross the blood brain barrier in normal mice, exhibit no acute toxicity, and act as HDAC inhibitors in the mouse brain. Importantly, our compounds increase FXN mRNA levels in the brain and heart in a mouse model for FRDA. In the present proposal, we plan to use synthetic methods to improve on our compounds in terms of pharmacological properties (improved brain penetration and metabolic stability), HDAC enzyme selectivity and efficacy. The effects of the inhibitors on FXN mRNA and frataxin protein will be determined in cell culture using a newly developed human neuronal cell model for this disease based on patient-derived induced pluripotent stem cells, and the genome-wide effects of the most active inhibitors will be assessed by DNA microarray analysis. Acute and chronic toxicity studies will be performed in normal mice, and mouse models harboring FXN genes with expanded GAA alleles will be utilized to determine whether HDAC inhibitors activate FXN gene expression in vivo, and relieve neurological deficits in these animals. The most promising compounds identified in these in vivo studies will be subjected to full pre-clinical evaluation in the hope of identifying a clinical candidate for human therapeutic trials.

Spotlight on Research Initiatives

Kyle Bryant Translational Research Awards

The Kyle Bryant Translational Research Award focuses on pre-clinical/translational or clinical research, advancing lead candidates into or through the research pipeline. In its third year, the proposals are only becoming more competitive and compelling. This year two grants were selected for the Kyle Bryant Translational Research Award, one of which is a clinical trial.

High-throughput meso-scale discovery of frataxin enhancers

Principal Investigator: Dr. Devin Oglesbee, Mayo Clinic, Rochester MN

This proposed study seeks to refine a sensitive assay that has been commercially validated to measure levels of frataxin protein in biological fluids and tissues, convert its reagents to be applicable to a high-throughput drug discovery assay, and complete a preliminary study of a chemical library in order to identify novel compounds that up-regulate or stablize frataxin levels in human cells.

An open label proof of principle study of resveratrol as a treatment for Friedreich ataxia

Principal Investigator: Dr. Martin Delatycki, Murdoch Children's Research Institute, Melbourne, Australia

There are currently no treatments proven to change the inexorable progression of this disorder. Since the identification of the causative gene in 1996 much has been learned about the underlying mechanisms in cells that cause the symptoms of this disorder. This has led to identification of a number of therapies proposed for testing as possible treatments for FRDA. One such group of therapies increase the level of the deficient protein frataxin. A medication called resveratrol increases frataxin levels in cellular models. In addition, it is an antioxidant and there is evidence that it is beneficial for diseases affecting the central nervous

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2010 Friedreich's Ataxia Treatment Pipeline

By Jennifer Farmer

Two of FARA's strategic research initiatives focus on the treatment pipeline. One initiative is to advance candidates on this pipeline, moving candidates from the research lab to clinical trials and ultimately to approved treatment. The other initiative is to support research that will bring new lead candidates to this pipeline.

FARA supports these pipeline initiatives in many ways.

- *Providing research grants to scientists who are working on lead candidates.*
- *Promoting collaboration with academic discovery scientists and pharmaceutical partners.*
- *Advocacy at a national/government level that increases opportunities and resources for individual scientists and pharmaceutical companies advancing treatments for rare diseases and promoting better, expedited regulation of the drug development and approval process.*

It is important to remember that clinical trials are still research experiments; while we are excited when a therapeutic candidate reaches the clinical trial stage of development (phases 1-3), it does not mean that it will be beneficial or successful as a treatment. Our colleagues in the drug development business often remind us that many drugs fail in clinical trials, so it is critical to have a robust treatment pipeline that is diverse in approach and deep in candidates. It is also important to remember that many drugs in clinical trials fail because not enough patients participate. That has not happened in FA and it is absolutely essential that FA clinical trials continue to attract enough participants to give each drug a valid trial.

Progress In The FA Treatment Pipeline

In 2010 there has been significant progress in the treatment pipeline.

Key advancements and new candidates

A phase II study of A0001 was initiated. A0001 is alpha-tocopherolquinone, a compound similar to Coenzyme Q10, which functions in the mitochondria. This study is examining the way glucose is converted to energy, which is often impaired in individuals with FA. This is the first time this candidate has been tested in individuals with FA. This study should be completed at the end of 2010 and results available in early 2011, when a decision will be made about proceeding to the next phase of clinical trial.

EPI-743 is a new candidate on the pipeline. EPI-743 is a new compound designed to be more potent than coenzyme Q10 and idebeneone, and to improve mitochondrial function. It is in phase IIB studies for inherited respiratory chain diseases (mitochondrial conditions) in acutely ill patients and in the late planning stages for a phase II trial for FA.

Resveratrol is not a new compound but new to FA. Resveratrol is found in the skin of red grapes. For those of you who are reading closely…yes, red grapes, red wine… unfortunately not enough is absorbed through red wine consumption! Resveratrol has been under intense investigation as a compound that could improve mitochondrial function and some studies suggest increased longevity, lowering of glucose levels, and anti-cancer activity. FARA has just funded an open-label, pilot study of Resveratrol.

At the end of the pipeline chart are several red bars that represent research designed to discover new therapeutic candidates. These are primarily high-throughput drug screens. A researcher develops an assay or a test in the laboratory. The assay is specific for function, i.e., improved mitochondrial function, increased expression of frataxin, etc… One such high-throughput assay measuring mitochondrial function in an FA model was developed by Dr. Robert Wilson at the University of Pennsylvania and has been used to screen hundreds of thousands of compounds. From this screening, supported by the National Institutes of Health (NIH), a promising structural candidate has been identified. With the additional assistance of a recently awarded FARA grant, Dr. Wilson is working with medicinal chemists to identify 2-5 compounds that are appropriate for testing in animal models of FA and pre-clinical development. Dr. Wilson's project is so promising that FARA is assisting him in the search for an appropriate pharmaceutical partner for drug development. Dr. Marek Napierala, featured scientist in this Advocate, has developed a different kind of assay that measures genetically influenced increases in frataxin protein. The NIH recently selected Dr. Napierala's assay to go through the same type of high-throughput screening program at the NIH as Dr. Wilson's assay. This allows access to one of the largest drug-compound libraries for testing, provides additional support through a large database of knowledge on the compounds, and assists with selecting lead candidates from the assay results. FARA is confident that these research projects will bring new specific lead candidates to our pipeline in the near term.

Completion of clinical studies

A pilot/phase II study of Chantix/Varenicline concluded earlier in 2010. While the initial case evidence of this drug was encouraging, this study was stopped due to concerns regarding safety and intolerability and insufficient evidence of efficacy. We are most appreciative of all the individuals and investigators who participated in this study. While the study did not prove a therapeutic benefit in individuals with

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Marek Napierala

Interviewed by Paul Marcotte

Marek Napierala, Ph.D., is an Assistant Research Professor with the University of Texas MD Anderson Cancer Center in Houston and one of the world's leading scientists studying Friedreich's ataxia. In 2009, he received a Kyle Bryant Translational Research Award for his proposal, "Crosstalk between microRNAs and iron metabolism in pathogenesis of Friedreich's ataxia." He is also currently working on identifying new compounds for the treatment of Friedreich's ataxia, and is one of the scientists planning the 4th International Friedreich's Ataxia Scientific Conference, which will be held in May 2011 in Strasbourg, France.

While still a student you began your Friedreich's ataxia research in Houston with Bob Wells, who is a former scientific advisor to FARA. How did that experience get you interested in FA?

Actually, I came to Dr. Robert Wells' laboratory in Houston as a young postdoctoral fellow, just about a week after my Ph.D. graduation. Already as a Ph.D. student at the laboratory of Dr. Wlodzimierz Krzyzosiak in Poland I was interested in the biology of DNA repeats including GAA sequences, the mechanisms of their expansion, and processes leading to the neurodegenerative diseases. Great work of Dr. Naoki Sakamoto, another postdoctoral fellow in Bob's laboratory, on DNA structures formed by GAA repeats, directed my interests into the FA field.

In 2007–2008, you received FARA's first New Investigator Award, which is awarded to outstanding new researchers who are dedicating themselves to FA research. Tell us about the research you did as a new investigator.

This was a very important moment for me. The New Investigator Award from FARA and Young Investigator Award from National Ataxia Foundation paved my path into the independent research in the area that was the most fascinating for me. Thanks to these Awards and great mentors I could develop my research program on uncovering molecular mechanisms of Friedreich's ataxia. I strongly believe that to be able to develop specific and effective therapies for Friedreich's ataxia, first we need to understand the underlying causes of the disease.

Last year you received a Kyle Bryant translational research award from FARA. What have you learned from doing this translational research?

In the project sponsored by Kyle Bryant Award, we identified a connection between deficiency of frataxin, expression of small regulatory molecules called microRNAs, and iron metabolism. MicroRNAs are a relatively novel class of very important modulators of gene expression. They are affected in several human neurological disorders like Huntington's, Parkinson's and Alzheimer's diseases. In Friedreich's ataxia we identified a subset of miRNAs that are changed in patient cells. Interestingly some of these miRNAs regulate expression of proteins crucial in maintaining iron homeostasis.

Can you tell us more about why microRNAs are important to FA research? Thus far we do not have any data related to the expression of microRNAs in FA cells. In order to fully understand the molecular mechanism of Friedreich's ataxia, it is important to analyze every aspect of the disease, including expression of regulatory molecules such as microRNAs. Our data suggest that problems with microRNA expression in FA may affect iron metabolism and participate in development of cardiomyopathy. Additionally, these small molecules are good biomarkers of the disease.

Your research has focused on genetic-based approaches to FA. One of your research projects has been to develop a high-throughput assay for use in drug discovery. Can you explain how your high throughput assay works and how it is important in screening new compounds?

Mutations leading to FA are unique among genetic diseases in that the correct genetic information is preserved in FA patients, but due to expansion of GAA repeats it cannot be expressed at sufficient levels. So far there is no effective treatment for Friedreich's ataxia. In order to identify drugs which may lead to the increase in frataxin levels, we will analyze several hundred thousands of molecules in the search for activators of frataxin gene expression. In order to conduct the screen of such a large number of compounds, we developed an assay based on a so-called reporter gene. In principle, this reporter gene mimics the frataxin gene in such a way that its expression is also inhibited by very long GAA repeats. However, in comparison to the frataxin gene, the changes in the expression of the reporter gene can be analyzed very precisely and efficiently using robotics.

Your assay was developed in part through grants from FARA, and more recently the National Institutes of Health (NIH) awarded funding for your assay to go through their HTS screening program at Scripps, FL. What will be happening at Scripps?

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The assay we developed will first be transferred to the Scripps screening facility and adapted to the high-throughput requirements. The settings and conditions differ significantly from our laboratory. The entire process needs to be ready for operation at the extremely small volumes in a completely robotic environment. We have already validated the system for its sensitivity and reliability in the laboratory settings. Now we are preparing and testing large quantities of material to be ready for HTS screening. The high-throughput screen — actually there will be two independent screens — will include more than 300,000 compounds.

What do you expect will come out of the research at Scripps?

The high-throughput screen should identify a few hundred compounds (potential leads) that would positively affect expression of our reporter minigene. These compounds will then be validated using three different secondary assays. This work will be conducted in our laboratory in Houston and will not require high throughput equipment. Potential candidate compounds will be tested in Friedreich's ataxia cell lines, both fibroblasts and neuronal cells derived from induced pluripotent stem cells. At the present time, we are learning how to efficiently obtain neuronal cells from pluripotent iPS cells to be ready for future testing of compounds identified in HTS. Hopefully at the end of the screening campaign we will identify 1 or 2 compounds, called probes, which can be further tested and possibly developed into the functional drugs.

You are currently serving on the Steering Committee for the 2011 4th International Friedreich's Ataxia Scientific Conference, which will be held in May in Strasbourg, France. FARA is one of the sponsors of the conference, which is drawing leading FA researchers from around the world. What do you expect the conference will accomplish?

These meetings are an excellent source of knowledge about FA research. Scientists from all over the world interested in FA gather and exchange their results and ideas, and start collaborations. The disease is dissected from all directions starting with basic, molecular mechanisms and finishing with its clinical aspects. For three days we talk and listen only about Friedreich's ataxia. Considering recent progress in the understanding of FA, this is going to be a very exciting meeting.

How long do you think it will be before there are effective treatments for FA?

This is a difficult question. Discovering, testing and approval of a new drug is a very long and difficult process. There are currently a few clinical trials for drugs which may at least partially alleviate the disease symptoms. Some very promising candidates, like HDAC inhibitors developed at The Scripps Research Institute, and Repligen, are in the advanced pre-clinical or early clinical phase testing. Developing a successful treatment for Friedreich's ataxia is likely to be a stepwise process with drugs partially correcting the disease symptoms. We also hope that sooner than later the progress in regenerative medicine will allow correcting the defects of this neurodegenerative disease. ■

FARA's Research Grant Program

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system. This open-label study of resveratrol in Friedreich ataxia aims to determine whether resveratrol increases frataxin in blood cells of individuals with Friedreich ataxia.

The Keith Michael Andrus Memorial Award

The Keith Michael Andrus Memorial Award was established as a tribute to Keith's legacy and to help improve the lives of others diagnosed with FA. This award is specifically designated for cardiac research that targets the understanding and/or treats the cardiac complications associated with FA. For example:

- *Exploring the underlying mechanisms of cardiac disease; e.g., testing hypotheses of mitochondrial dysfunction in FA that might lead to cardiac phenotype in patients;*
- *Clinical studies that further elucidate the cardiac phenotype, natural history, and biomarkers, or*
- *Prospective treatment trials that will allow for the establishment of informed treatment recommendations.*

The cardiomyopathy of Friedreich's ataxia

Principal Investigator: Dr. Arnulf Koeppen, Albany Research Institute, Albany, NY

Heart disease ("cardiomyopathy") is very common in patients with Friedreich's ataxia (FRDA) and is often the cause of death. The "ejection fraction" of the left heart chamber (ventricle) declines as the disease progresses and ultimately does not meet the demands of organs such as kidney and brain. Heart disease in FRDA advances

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FARA's Research Grant Program *(continued from p. 7)*

independent of the nervous system disorder (ataxia). This independently of the nervous system disorder (ataxia). This research focuses on the role of iron in FRDA in the main "working" heart muscle and the cardiac conduction system. This system consists of specialized muscle fibers that deliver excitatory impulses to all chambers of the heart. Normally, right and left ventricular walls and interventricular septum contract in synchrony to generate a powerful heartbeat. The triggering impulses travel in two steps from the sinoatrial node in the right antechamber (atrium) to the atrioventricular node at the junction of atria and ventricles. From the atrioventricular node, additional unique fibers constituting the "bundle of His" conduct impulses in a rhythmic fashion to the main heart muscles. Friedreich's ataxia is due to an inherited loss of a small protein, "frataxin" (named after Friedreich's ataxia). The hypothesis is that frataxin deficiency causes diffuse or localized iron excess in the heart; and that the metal is toxic to the main "working" heart muscle fibers and the cardiac conduction system. The investigation will use heart tissues of deceased patients with FRDA whose families have donated organs for FRDA research. The two main methods are (1) X-ray fluorescence of heart tissue to detect and measure iron in the main muscles and the fibers of the conduction system; and (2) advanced slide techniques to visualize iron, the iron-responsive proteins ferritin and mitochondrial ferritin, and proteins of the conduction system. The clinical relevance is insight into iron in FRDA cardiomyopathy to improve decision-making about the potential benefit of chelation therapy. ■

4th International Friedreich's Ataxia Scientific Conference, May 5-7, 2011

Institute of Genetics and Molecular and Cellular Biology (IGBMC) Strasbourg, France

The IGBMC has generously offered to host this meeting and Dr. Hélène Puccio has volunteered to serve as Chairperson for the conference. The European Friedreich's Ataxia Consortium for Translational Studies (EFACTS) and Euro Ataxia groups elected to hold their annual meetings in concert with this conference on May 8, 2011. Also, many of our advocacy partners such as Ataxia UK, FASI, FARA-Australasia, GoFAR, AFAF, and others committed their support.

Goals and Objectives:

- Bringing together basic, translational and clinical scientists working on FA
- Sharing new data/research findings, hypotheses, and unpublished work
- Promoting collaborations and knowledge sharing to advance progress in FA research
- Supporting students and post-doctoral scientists who are developing their research careers in FA

Important Dates:
December 15, 2010 – Abstracts due
January 15, 2010 – Registration closes

More info:
www.curefa.org/conference.html or e-mail
conference2011@curefa.org

Autopsy and Tissue Donation Program

Learn more about autopsy and tissue donation for research. FA is a very complex disease that affects spinal cord, nerves, brain, heart, bone, and the insulin-producing cells of the pancreas. Through the Autopsy and Tissue Donation Program, researchers are able to conduct a rapid autopsy and harvest important organs to study, leading to improvements in the diagnosis, treatment, and care of others with FA. Many of our recent breakthroughs are based on research made possible through tissue donations from FA families.

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

Dr. Arnulf H. Koeppen
VA Medical Center
113 Holland Ave
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Tel. 518-626-6391; 518-626-6377; FAX 518-626-6369;
e-mail: **arnulf.koeppen@va.gov**

Parent Liaison

As a parent who has gone through the tissue donation process, Mary-Lisa offers her compassion and wisdom and 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. To learn more, contact:

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From the Sunnier Side: Perspective from a Wheelchair User

By Robbi A. Van Schoick, MPH

Robbi A. Van Schoick was born November 3, 1979 in Lubbock, Texas to Nelda and Bob Van Schoick. The family (her parents, Robbi, and her two sisters, Becca and Katie) lived in Memphis, TN when Robbie first started showing symptoms of Friedreich's ataxia at age five. Her mom, a physical therapist, began to notice her widespread gait, highly arched feet, and lack of coordination, but without genetic testing, her suspected diagnosis of Friedreich's ataxia was not confirmed. When she was in the 4th grade, the family moved to France, where she lived for 18 months. When the family moved to New Jersey in her 5th grade year, Robbi's younger sister, Becca, began to show symptoms of FA as well. Both girls were using wheelchairs when the family moved to east Texas in 1994 to escape the harsh northeast winters.

Robbi graduated from Bullard High School in the top 10 percent of her class, and attended Southern Methodist University in Dallas, TX as a psychology major until her father's job mandated a move to Georgia in 2001. She continued her BS at the University of Georgia, where she won a psychology department award for the best undergraduate paper on the ameliorating effects of dietary behavior on FA. She graduated cum laude from the University of Georgia in 2003, and then went back to obtain a master's degree in public health, which she pursued one class at a time. Robbi received her MPH in May 2010. The following is an excerpt from her master's thesis.

Perhaps we are driven by evolutionary forces to observe people who are physically atypical with more intensity than we do the average passerby. Or perhaps, our ubiquitous desire for knowledge, for understanding, leads us to hold our gaze on those who appear, in some way, novel. Being the information-seekers we are, it is inherent for us to not only observe strangers, but to find a plausible explanation for any given problem, including the enigma of why someone who looks healthy and without obvious deformity would be using a wheelchair. In my nearly 20 years of experience as a wheelchair user due to a rare degenerative disease called Friedreich's ataxia, I have grown accustomed to both types of behavior; people taking notice of me and obviously trying to interpret my situation. Whether we want to admit it, each one of us takes more notice of people who are physically different, in terms of race, ethnicity, and disability. I wholeheartedly believe that observing someone less ordinary is merely a natural corollary of human nature, rather than grounds for belligerence or ill-wishes.

As for the judgment to which I have grown accustomed, the overwhelming majority of people I meet tend to assume that because I look "normal," but use a wheelchair, I must have the typical symptoms of a person with paraplegia: full motor control, no hearing impairment, no difficulty articulating, and perhaps the most problematic in terms of expectations at work or school, a normal energy-level. It is understandable that some wheelchair users, including myself, are often lumped together to form a homogeneous mass of people with the same symptoms. Albeit reasonable, the judgment is as meaningful and apropos as, if I may use the familiar analogy, comparing apples to oranges. Indeed, society should endeavor to look beyond the wheelchair to acknowledge the differences of the individual person who uses it, rather than continuing to automatically arrive at "paraplegic" when a healthy-looking wheelchair user comes into view. Long ago, I stopped counting the number of times strangers have approached me with quizzical expressions and asked me why my legs don't work, or, specifically, about my "accident." Apparently, the tendency is not sex-specific, as men who use wheelchairs due to the disease I have report the same reactions when in public. However, men are occasionally asked if they are veterans, wounded in combat. Why else would an average-looking man be using a wheelchair?

For some, the tendency to judge healthy-looking wheelchair users as having equivalent impairments may be borne out of discomfort with physical abnormalities; for others, it may stem from simple unawareness of medical problems. To those who find the reaction offensive, I argue that there is no reason to waste time and energy placing blame. Rather than feeling angry or affronted that many people see someone healthy-looking using a wheelchair and assume the cause to be paralysis, I encourage these wheelchair users to respond with discerning, knowing tolerance. Without a doubt, patience is as requisite to life with a disability as oxygen is to plant life.

I will admit to being rather disgruntled at a few adults who have blatantly stared at me in public, however my typically short-lived moments of frustration have rarely led me to feel like a helpless victim of out-group stereotyping. Rather than feeling like a specimen to be scrutinized by the public or an attention-grabbing exhibit in a freak show, I try my best to be good-natured and patient with people and to remain cognizant of the belief that looking at someone extraordinary is only innate.

Continued on p. 11

Performance Award Center, Med-Pharmex Named 2010 Partners of the Year

By Felicia DeRosa

This year FARA proudly presents our 2010 Partner of the Year Award to Performance Award Center (PAC) and Med-Pharmex. Long-standing generous supporters of FARA through the Van Schoick Benefit Bash, both companies decided to get more directly involved in supporting FARA. In 2008, the CEO of PAC, Jerry Russell, and the CEO of Med-Pharmex, Vince Palasota, launched their own event in Texas. The Lonestar Benefit Bash follows the golf tournament and dinner/dance party model initiated by the Van Schoicks in Georgia, bringing it to a whole new group of supporters in Texas, many of whom are learning of FA for the first time through this event. Key staff members of both companies gave their personal time to organize the last two annual events and are in the process of planning the third for March 25, 2011. Next year's event will take place in conjunction with Ride Ataxia Dallas on March 26. The Lonestar Benefit Bash has raised over $125,000 in support of FARA's mission and the organizers have also made countless additional contributions to FARA through the Van Schoick Bash.

This year PAC also brought its professional talent to FARA with the donated development and launch of the FARA web store: www.curefastore.com PAC specializes in promotional products and web stores for the purpose of increasing organization brand awareness; PAC's clients include Snapple, Marriot, Chili's, and Nissan. This store provides the FA community with a central online portal to order items for events and fundraising at a reduced price, ensuring brand consistency at events across the country. For events such as Team FARA in which team participants run in a jersey with FARA's name and logo, as well as grassroots events hosted by families who give out awareness bracelets, items from the web store help raise critical awareness for this rare disease.

FARA's partnership with PAC and Med-Pharmex started because the Van Schoick family inspired these companies to give to their fundraiser. As a result they not only gave resources but also their time and their talent to raise awareness and support for FA research. Mr. Russell and Mr. Palasota state on their event website, "We have gotten to know Bob [Van Schoick] and his family and have decided that this rare disease deserves attention and support." Thank you Mr. Russell, Mr. Palasota, PAC and Med-Pharmex for recognizing the needs of a rare disease research initiative and growing the community of support behind FARA!

FARA Partner of the Year Selection Criteria

- *Leadership and/or employees/constituents within the partner organization directly support 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* ■

Jerry Russell, Robbi Van Schoick, Ron Bartek, Becca Van Schoick, and Vince Palasota at the 2008 Van Schoick Benefit Bash.

The Lonestar Benefit Bash 2008 was created by Partners of the Year winners Performance Award Center and Med-Pharmex.

AAI Grant Recipients Announced

FARA, in partnership with the Christopher and Dana Reeve Foundation and The FA Project, congratulates Jennifer Deneau of Michigan, and Nick Carbone of Massachusetts as recipients of the 2010 Ataxian Athlete Initiative (AAI). AAI seeks to empower ataxians by funding the purchase of adaptive cycling equipment through a competitive application process. To learn more, visit: **www.rideataxia.org/AAI_2010.pdf**

Working Steadily Toward a Cure
Carrie Laird, Bakersfield, CA

By Marilyn Downing

Carrie's motivation to raise money for a cure is her son, Jerod, who was diagnosed 10 years ago with Friedreich's ataxia. Now 23, he is working toward a business degree in college. Carrie immediately wanted to begin raising money to find a cure, but Jerod was not prepared for the attention, so it was a few years before the fundraising whirlwind began at the Laird house.

Carrie told Jerod they would do something small. Little did he know that prior to one of the first events there would be TV trucks in front of the house with reporters ready to interview him to find out how he felt about having FA! Since then, the Lairds have organized races, walks, climbs, barbecues, jog-a-thons, Outback luncheons, raffles, Macy's "Shop-for-a-Day" ticket sales and "Stuff-on-a-Stick" food booths. Their amazing committee meets regularly year round and includes many of Jerod's generous friends.

The Lairds' local community now is very familiar with Friedreich's ataxia and as Carrie says – people are more willing to give when the disease is recognizable and particularly when they know someone with the disease. Thank you, Jerod, for stepping up to the plate! Recently, after Jerod spoke at a school assembly, one elementary student told local media that he wanted to get involved so that he "could help Jerod walk."

Raising funds to fuel the research and raising awareness about FA are Carrie's passions. Jerod appreciates all that's been done in his honor, but more than anything he is anxious to find a cure so that he can have his mom back! ■

Living with FA *Continued from p. 9*

Much like we can positively or negatively construe people looking intently at someone physically unusual, we can choose the way in which we interpret people assuming that the average-looking person who happens to use a wheelchair must have simply had some sort of accident. Some may choose more negatively-toned explanations, such as most people are too uncomfortable with "messy" reasons for using a wheelchair, so they instantly call upon the familiar, clear-cut explanation that the person's legs are paralyzed. Personally speaking, I tend to dwell on the sunnier side of most philosophical issues, and on this one, I, again, believe in the enduring strength of human nature. In other words, I think it is perfectly natural to look to the most obvious explanation for this quandary of a "normal", healthy-looking person using a wheelchair. Moreover, I find optimism in the suggestion itself, because to assume one has lost mobility in some sort of accident is to assume he or she once had the ability to walk. I have heard of people in my situation who have negative reactions to such encounters, and I urge them to reevaluate the deeper meaning of strangers' common assumption. Hopefully, even a pessimistic thinker might see the other side. ■

2010 Friend and Ally Award Winners

by Jamie Young

Established in 2009, the FARA Friend & Ally Award honors volunteers in the Friedreich's ataxia community who have demonstrated exceptional effort in support of FARA's mission—to treat and cure FA. It will take the united efforts of many to find treatments and a cure; therefore, we value each one of our "Friends" and "Allies." The recipients of the 2010 FARA Friend and Ally award are Mary Hallett-Brown, Blair DeSaw, and Julie Robertson. All three award winners have helped us nurture and grow our community through outreach.

Mary Hallett-Brown—Facebook

A volunteer with FARA for over three years, Mary Hallett-Brown hosts her own family fundraising events, volunteers at Ride Ataxia Philadelphia, and keeps FARA connected to our community through Facebook. FARA's community and collaborations stretch worldwide, so it takes communication tools like Facebook to keep everyone informed of opportunities and FARA news in a timely fashion. Mary goes above and beyond not only by posting information that's supplied by FARA staff, but also by suggesting new relevant content for posting. Mary has helped create an online community for FARA families to stay connected and to know that they are not alone in the battle against FA.

Blair DeSaw—FARA Websites

Blair DeSaw has been volunteering as FARA's webmaster for the past two years. Blair manages the content for FARA's main curefa.org website, as well as for the Ride Ataxia and FARA Energy Ball event sites. Blair's dedication to FARA communications is manifested by real time, up-to-date website content, including new scientific publications, FARA events, and FARA news. An informative and functional website allows us to communicate with countless individuals—within the FA community and potential partners.

Julie Robertson—Outback Steakhouse

Julie Robertson, of OSI Restaurant Partners, facilitates events and outreach programs to benefit FARA, including numerous Outback luncheons and the Heineken with a Heart series. Such events raise critical funds and awareness for this rare disease, and provide an opportunity for FA families to come together and be an empowered part of funding and advancing FA research. Julie has also devoted a great deal of personal time to play a key role in FARA's marquis fundraisers—Ride Ataxia and the FARA Energy Ball.

Thank you Mary, Blair, and Julie for being committed Friends & Allies to FARA and for your support of our mission to treat and cure FA together! ■

Volunteers Blair DeSaw and Mary Hallett Brown

Volunteer Julie Robertson, Outback Steakhouse

The Ataxian

The Ataxian Documentary captures the journey of Team FARA in the Race Across America (RAAM) and is in post production right now. Filmmakers Kevin Schlanser and Zack Bennet are weeding through 200 hours of film to create the full-length documentary.
http://theataxian.com

The FARA Advocate is brought to you by:

Contributors: *Ron Bartek, Kyle Bryant, Felicia DeRosa, Marilyn Downing, Jennifer Farmer, Dr. Bronya Keats, Paul Marcotte, Jamie Young*

Editor: *Karen Smaalders* ***Design/Layout:*** *Anne Myers*

Ride Ataxia Program Update

By Kyle Bryant

It's not supposed to be easy. Sam knows the meaning of these words. She lived these words every time she got on her bike to train for Ride Ataxia Philadelphia. Sam stretched her budget a little bit and dove in head first when she decided to buy her handbike at the beginning of August – only two months before the ride. Sam knew it was not going to be easy, but she also realized that she is not on this earth, in this fight against FA, merely to survive. She is here to make an impact, to conquer FA. Sam trained for two solid months in order to conquer the impossibly steep hills of Pennsylvania on the 10-mile course at Ride Ataxia Philadelphia. Sam struggled up the hills all day, refusing help because she was determined to make it under her own power.

Sam with her Mom, Mary and sister Alex after completing the 10-mile route at Ride Ataxia Philadelphia.

This story repeated itself many times in all the 2010 Ride Ataxia events. The rides featured challenging courses for all levels in Florida, Northern California, Philadelphia, and Southern California. These rides involved a total of 750 cyclists and countless volunteers, who collectively raised over $350,000 for FA research. Each Ride had a character of its own, such as the formidable NorCal Ride which challenged 2-day riders to 8,000 feet of climbing in 100 miles, or the Tampa ride, which was a scenic roll in the Tampa sun.

Outback Steakhouse came out in full force at each Ride Ataxia event in 2010, offering incredible post-ride meals and volunteering wherever needed. At the Philadelphia ride, cyclists were treated not only to post-ride food by Outback, but also by Bonefish Grill and Carrabba's Italian Grill.

In 2010, each participant took on a challenge: at times biting off a little more than they thought they could chew, but in the end each person acknowledged that

Keith O'Brien with Kyle Bryant at Ride Ataxia Philadelphia.

it's not supposed to be easy. We are not here for a Sunday roll in the park; we are here to cure a disease.

As Sam crossed the finish line triumphantly under her own power, and she was greeted by a huge crowd of teammates and supporters, she realized that with every turn of the crank that day she was raising awareness, inspiring people to give to research, and getting us all closer to the cure.

The Future

In 2011, Ride Ataxia again will feature rides in Florida, Northern California, Philadelphia and we will add a ride in Dallas. The Dallas ride will be completed with the key partnership of Outback Steakhouse and will take place in conjunction with The Lonestar Bash Golf Tournament fundraiser. The Lonestar Bash Golf Tournament is hosted by FARA's 2010 Partners of the Year Performance Award Center (PAC) and Med-Pharmex.

Another change for 2011 is that the Florida Ride will relocate from Tampa to Orlando to facilitate a partnership with Big Cat Human Powered Vehicles (catrike.com). Big Cat Human Powered Vehicles has a strong presence in the cycling community, particularly in Orlando.

If you are in the fight against FA. . . If you want to make an impact. . . Join the Ride Ataxia team in 2011. Challenge yourself and help turn the crank towards a cure.

Find out more by checking at **www.rideataxia.org**, search "ride ataxia," and "like" us on Facebook or follow us on Twitter at **twitter.com/rideataxia**. ■

Ride Ataxia 2011 Dates

Dallas – Lewisville, TX	March 26, 2011
NorCal – Sacramento, CA	May 14, 2011
Philadelphia – Limerick, PA	October 9, 2011
Orlando, FL	November 2011*

**Date to be confirmed*

FA, the FA study investigators continue to evaluate this pathway and the drug continues to be investigated in some of the other ataxias.

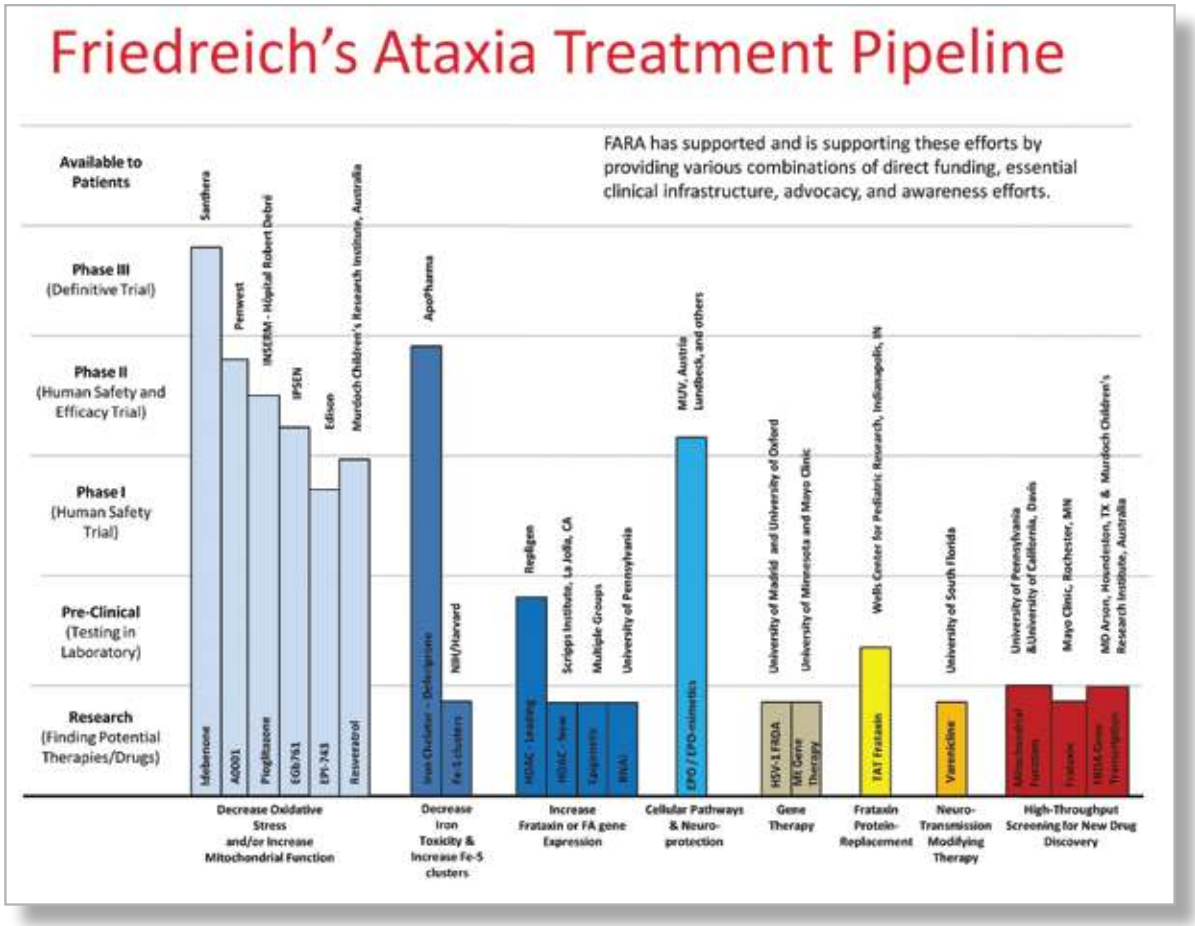
Ongoing studies

A phase II, proof-of-principle study of Pioglitazone is ongoing in France. Pioglitazone is a drug that has been developed for the treatment of diabetes but is also known to induce enzymes involved in mitochondrial metabolism and the recruitment of antioxidant enzymes. Pioglitazone has been shown possibly to act on neurodegeneration in humans and animals models. This trial is expected to conclude in 2011.

A phase II study of a compound called Lu AA24493 or cEPO, a novel carbamoylated form of human erythropoietin (EPO), has been initiated in Germany, Austria and Italy. You may recall that, in earlier studies in FA, it appeared that EPO may act to increase frataxin protein levels but also to increase red blood cells. This modified form of EPO (cEPO) is thought to have fewer haematopoietic effects (so would not increase red blood cells) but to maintain the tissue-protective effect (possibly by increasing frataxin protein levels). The primary objective of the study is to evaluate the safety and tolerability in patients with Friedreich's ataxia. This study is expected to conclude in 2011.

The FA treatment pipeline is updated on a regular basis on the FARA website. There is also a more detailed description of each of the lead candidates and the status online at: http://www.curefa.org/pipeline.html.

Your participation is critical. Please ensure that FA patients sign up on FARA's patient registry (www.curefa.org/registry) so they can be notified regarding clinical trials for which they appear to be eligible. This website 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 is absolutely necessary for success, and they are looking to us for that support. ■



The FA Treatment Pipeline is a visual tool for communicating the progress of research and development on lead therapeutic candidates. Along the horizontal axis lead candidates are grouped based on mechanism of action or approach to treatment, i.e., where or how each might work in the cell, technological approach, or problem being addressed. The vertical axis indicates the stage of the research —where the candidate is in development. The first two stages, research and pre-clinical, take place in the research laboratory, and represent early discovery and development. The stages "phase 1" through "available to patients" are phases of clinical trials/studies.

Amy Hawk, Outback Pro-Am tournament director, presents a check to FARA Executive Director Jen Farmer and President Ron Bartek at the FARA Energy Ball.

Energy Ball Raises Record Amount to Cure FA

By Felicia DeRosa

Small circular ice sculptures perched in the middle of the tables reflected light in the ballroom as over 850 guests arrived at A la Carte Event Pavilion for the second annual FARA Energy Ball benefitting the Friedreich's Ataxia Research Alliance (FARA) and the University of South Florida Ataxia Research Center (USF-ARC). The bright colors of the event logo danced with the light and embodied the vibrant energy of the guests at this sold-out venue.

The FARA Energy Ball marked the highest attended 2010 Tampa fundraising event and was the culmination of a week of activities starting with *"Cultivating the Cure,"* an educational research symposium at USF-ARC. Symposium attendees heard updates on FA research from FARA's President, Ron Bartek, and Executive Director, Jennifer Farmer. Dr. Joel Gottesfeld from Scripps Research Institute presented an update on HDACi; Dr. Guy Miller of Edison Pharmaceuticals talked about a new compound being tested in mitochondrial diseases- EPI-743; and Dr. Theresa Zesiewicz and Dr. Lynn Wecker of USF spoke about the potential role of nicotinic receptors in ataxia. Other activities leading up to the ball included a co-ed softball game, a tennis tournament, and a Martinis & Poker Patron Party.

The FARA Energy Ball featured a four-course meal and live and silent auctions with something for everyone including get-aways, dining, jewelry, autographed memorabilia, art, spa packages, and event tickets. During dinner, Dr. Michael Spino from Apopharma educated guests about the iron chelation effort in FA. Following dinner, Sister Hazel got the crowd on its feet and continued to raise the level of energy in the room performing favorites like "*All for You*" and *"Change Your Mind."* Due to the event committee's creativity and meticulous preparation as well the generosity of sponsors, guests, and in-kind donors, the 2010 FARA Energy Ball was an amazing success, raising $1.3 million (gross proceeds). We are excited to announce the dates for the 2011 FARA Energy Ball, scheduled for August 25–27. A special thank you to our sponsors:

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2010 Events	Event Planners	Location	In Honor Of
NORTHEAST			
Play for FA- Family Fitness Night	Kim Bellnier	Rochester, NY	Alec and Colin Brown
*Sorority Sisters Fundraiser	Kathleen Zanelli	UMass-Darmouth	Tiffany Gambill
*Concert for a Cure	Bobby Hughes	Herndon, VA	Alexa Early
*Flatbread's Charity Night	Erin O'Neil	Bedford, MA	Erin O'Neil
*Maul Family Event- Chinese Auction	Tricia, Tara & Chris Maul	Machias, NY	Tricia Maul and Tara Herman
*Holly's Hope Golf Tournament	Gary Dempsey	Bedford, NH	Holly Franz
*Seaside Stride	Karen O'Brien	Tom's River, NJ	Keith and Dylan O'Brien
Ham Supper & Walk	Dennis Wood	Jonestown, PA	Dennis Wood
Backyard Bash	Francine Welsh	Harrisburg, PA	Brendan Welsh
Fuzzy Buzzy Golf Tournament	Paul Stanieich	Windham, NH	Erin O'Neil
Olsen Golf Tournament	Dan Olsen	Monroe, NJ	Dan Olsen
3rd Annual McDonnell Music Day for FA	Dylan McDonnell	Queensbury, NY	Dylan McDonnell
Team Donovan Triathlon	Norm & Debra Simpson	Rye, NY	Donovan Simpson
*Hopkins Annual 5K Race for FARA	Katie Hopkins	Briston, RI	Grace Hopkins
Krivinko Classical Piano Concert	Nancy Krivinko	Pittsburgh, PA	Lauren Krivinko
SOUTHEAST			
VanSchoick Golf Tournament & Benefit Bash	Nelda VanSchoick	Athens, GA	Robbi and Becca Van Schoick
*Swing Away at FA	Candy Stacks	Cumming, GA	Hannah Stacks
*Athens Half Marathon	Katie VanSchoick	Athens, GA	Robbi and Becca Van Schoick
*BlackFinn Restaurant Opening	BlackFinn	Jacksonville, FL	FARA
*Vida Restaurant Opening	Vida	Charlotte, NC	FARA
*2nd Annual Harry Hunt Rusty Nail Invitational Golf Tournament	Jennifer Shaw	Howey-in-the-Hills, FL	Tara Ryan
*Clemson Sports Medicine's Strive for 5- 5k for FARA	Catherine Sullivan	Clemson, SC	FARA
Ace for the Cure	Sandy Callaghan	Tampa, FL	FARA
MIDWEST			
Luebbe Dodgeball & Dinner	Tammy Luebbe	Liberty Township, OH	Evan Luebbe
*Beat Ataxia Blues	Linda Janota	Chicago, IL	Alena and Alisa Wolfson
Peters' Hole Out for a Cure	Rick Peters	Peoria, IL	The Peters Family
*Jack's Ride for a Cure	Ruth DeWitt	Howell, MI	Jack DeWitt
WEST			
*First Annual Helms Fundraiser for FA	Jen and Jason Helms	Missoula, MT	Dylan and Sienna Helms
Martinez Ride Ataxia Party	Angela Lacativo-Green	Martinez, CA	Kyle Bryant
Prater Family Ride Ataxia Party	Becky Prater	Chico, CA	Kyle Bryant
Truckee Ride Ataxia NorCal Party	Neal Lacativo/ Bryant Family	Truckee, CA	Kyle Bryant
*Northern California Backyard Party	Lisa Frohling/ Diane Bryant	Auburn, CA	Kyle Bryant
SOUTHWEST			
Lonestar Golf Tournament	Jerry Russell & Vince Palasota	Dallas, TX	Robbi and Becca Van Schoick
Race 4 Results	Carrie Laird	Bakersfield, CA	Jerod Laird
*Century 21 King Charity Golf Tournament	Carolyn & Brian Lamascus	Ontario, CA	Joshua Lamascus
Stephanie's Hope	Sharon Magness	Valencia, CA	Stephanie Magness

**-Indicates first-time fundraiser for Friedreich's ataxia.*

2010 TEAM FARA Events	Event Participants	Location	In Honor Of
Cycling			
Race Across America	Kyle Bryant, Sean Baumstark, John Lockwood, Mike Mellott	Oceanside, CA- Annapolis, MD	FARA
Ironman			
Hawaii Ironman World Championships	Rachel Main	Kona, Hawaii	Adam & Sarah Main
Marathon Series			
Atacama Desert Run	Marilena Wilkinson	Chile	Veronica Pepe
Hiking/ Mountain Climbing			
Fool Killer Climb	Tom Hopkins & Family	White Mountains, NH	Grace Hopkins
Adirondack Great Range Hike	Mike DelSignore	Adirondack Mountains, NY	Dylan McDonnell
Running			
ING NYC Marathon	Sally Braid, Tim Williams, JP Swanson, Dan Pollner, & Eric Gies	NY, NY	Donovan Simpson, Kyle Waterman, Laura & Sara Ferrarone
Chicago Marathon	John Lagedrost & Friends	Chicago, IL	Joanna Lagedrost, Carli Hanson, Kati Hook
California International Marathon	Crystal Wade	Sacramento, CA	Kyle Bryant
Country Music 1/2 Marathon	Katie VanSchoick & Kyle Kilch	Nashville, TN	Robbi and Becca Van Schoick
Athens Half Marathon	Katie VanSchoick & Friends	Athens, GA	Robbi and Becca Van Schoick
Philadelphia 1/2 Marathon	Jen Farmer, Felicia DeRosa, Jamie, Tara & Andy Young	Philadelphia, PA	Emily Young
White Rock 1/2 Marathon	Tiffany Carroll	Dallas, TX	Madison Hopper
American River Parkway1/2 Marathon	Breanne Moen	Sacramento, CA	Rachel Gill
San Francisco 1/2 Marathon	Mark Bruemmer & Outback Team	San Francisco, CA	FARA
Broad Street 10 miler	Jen Farmer	Philadelphia, PA	FARA
Emerald Across the Bay 12K	Kate Walsh & Stephanie Hollingsworth	San Francisco, CA	Their Dad
Olympia Team FARA - Bank to Bay	Donna Morgan	Tacoma, WA	Gavin Morgan
Triathlon			
FA Moms Triathlon	Louise Chalcraft & Sandy Lane	Seattle, WA	Chelsea Lane and Joshua & Leah Chalcraft

2010 Outback & FA Family Events	In Honor Of
Outback Luncheons	
White Plains, NY	Donovan Simpson, Kaela Golanec, Albert Richard
Springfield, VA	FARA
Springfield, NJ	Keith and Lindsay Dalton, Connor Sweeny
Bakersfield, CA	Jerod Laird
Rohnert Park, CA	Brianne Konanz
Tacoma, WA	Roman DiCroce
Maumee, OH	Zach Zies

**Special thanks to Heineken, Outback and all participating FA families for the Heineken with a Heart series in Baltimore, MD, Royersford, PA, Chicago, IL, Tempe, AZ, and Atlanta, GA.

***Special thanks also to the DC Regional Outback; Ridge Sink and the Jacksonville, FL Outback; Tim Block and the Carolinas' Outback and Ron Duckstein and the Yorktown Lions Club for their fundraising golf tournaments for FARA.

Thank you to all of our FA families and friends for hosting fundraisers to raise awareness and support research advancement for FA. At the close of September 2010, these volunteers had collectively raised half a million dollars in support of FARA's mission to treat and cure FA.

Eilish, Francine, Brendan, and Pete Welsh host their annual Backyard Bash in Harrisburg, Pennsylvania.

Grace Hopkins poses with her family for a post race photo after the Annual 5K Race for Grace in Bristol, Rhode Island.

O'Neil family and friends having a great time at the Fuzzy Buzzy Golf Tournament in Windham, New Hampshire.

FARAmones and their supporters enjoying the day after the Chicago Marathon (L-R): Margaret Hay, John Lagedrost, Andrew Kustusch, Doug Worrall, Julia Lagedrost, Doug Finck, Carli Hanson, Kati Hook, Tom Trovinger, and Joanna Lagedrost.

Welcoming the crowd at the Holly's Hope Golf Tournament in Bedford, New Hampshire.

Conner Sweeny and Kyle Bryant hang around at the Springfield, New Jersey Outback Steakhouse event.

John Ryan, Dylan McDonnell, Lauren Williams, Tania Solange, and Terry and Dave McDonnell at the 3rd Annual McDonnell Music Day to Fight FA in Queensbury, New York.

Team Donovan gets ready for the Jarden Westchester Triathlon in Rye, New York.

Roman DiCroce and his best friend Jacob enjoy a delicious dinner at the Tacoma, Washington Outback Steakhouse event.

Great crowd at the Swing Away at FA Wiffle Ball Tournament and Family Fun Day in Cumming, Georgia.

Hannah Stacks, along with Laura Beth Jacquin and Jon Zilles, give the ceremonial first pitch at the Stacks Family Wiffle Ball Tournament.

Tom, Claire, and Francis Hopkins along with Webster Barrett on their Team FARA 2010 FoolKiller Climb.

Jack Dewitt leads his Ride for FA in Howell, Michigan.

Rick's Chicks help the golfers at the Peters' Hole Out for a Cure in Peoria, Illinois.

Vic Lafita of Kraft Foodservice with his daughter Alyssa (a volunteer from Delta, Delta, Delta Sorority) show off an adorable puppy in the silent auction at FARA's Energy Ball.

Two of FARA Energy Ball's co-chairs, Chris and Sandi Sullivan.

MEMORIALS

From January 2010 to October 2010, FARA has received nearly $100,000 in memory of the following individuals:

Alfredo Ramilo Acosta, John Andresen, Keith Andrus, Gustav Angantyr, William Austin, Paige Baker, Helen Barnett, Thomas Barnett , Jenny Bates, Raymond Bentley, Verla Bryant, Dick Buchanan, Camden Calbert, Pasquale Caruso, Joshua Chalcraft, Delores M. Cusick, Kevin Finnerty, Paul Flippo, Dallas James Gendall, Ronald Heuer, Lal H.Hingorani, Marcelino Huerta III, Emmabelle Innes, John Maxwell Jacquin, Mary Kime, Rosemary Kobler, Mary Lawson, Dave Lewis, Roberta Jean Lortscher, Janie Lowman, Sadie Magaro, Kevin Maher, Gioconda (Jackie) Martignetti, Robert May III, William May, Eleanor McNally, Nancy Mosier, Assunta Mullaney , Rev. J. Brendan Nally, Carmel O'Connor, Aubrey Olson, Nick Olson, Eileen Parsons, Gordon Peters, Jennifer Poplinger, James M. Powers, John Justin Quigley, Elena Raymond, Jeff Rosenkranz , Merrill Rushin, Eula Savoie, Amanda Sutton, Terri Stone, Owen Synder, Ron Velich, and Barbara Wood.

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.

Bonefish Grill Give, Get, and Win Sweepstakes

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Help FARA win:

Purchase $100 in gift cards & receive a $20 bonus card, which gives you a chance to win an additional $50 online that can be donated to FARA.

Visit **www.bonefishgrill.com** to vote for FARA (you can vote every day) and help FARA win $5,000.

Contact Us

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