

## From the Executive Director



### Shaping FARA for Success

By Jennifer Farmer

FARA's leadership recognizes the need to expand, grow and nurture our fundraising efforts to meet the growing momentum and number of research opportunities towards finding treatments – giving us all a sense of urgency and conviction. I am launching this regular column in The Advocate so that I can share with you information about various aspects of FARA's growth including strategic initiatives, operational issues, financial status, stewardship of FARA funds, and changes in leadership and staff. This first article focuses on 2009 initiatives to grow fundraising and development opportunities such as annual donations, events, and sponsorships. This expansion is driven by our need to fund not just more research, but much more expensive translational and clinical research. Several other articles in this issue give you a good summary of our research grant program and progress in 2009.

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## European Union (EU) to Fund FA Research

The EU recently informed the European Friedreich Ataxia Consortium for Translational Studies that it will fund the Consortium beginning in April 2010. The Consortium consists of 13 sites in seven European countries and is coordinated by Dr. Massimo Pandolfo. The funding level is being negotiated based on the Consortium's request for 6 million euros over a period of four years. (Details in next issue of The Advocate.)

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## Grassroots Fundraising



*The Rupel family (Katie, Matt, Bart, Brenda) at their FA-ITH event, with beautiful handmade quilts for the auction serving as a backdrop.*

## Having Faith and Having Fun is Key to Success of Rupel Family's FA-ITH Event

By Marilyn Downing

"It's about having FUN," Bart Rupel explains. The Hawaiian theme, the auctions (silent and live), the music (Bart's brother's band donates their special touch), and even the

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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.



### Everyone Needed to Help Fund Search for a Cure

*Dear FARA Friends and Supporters, 2009 has been a year of milestones for FA research and FARA. A record number of FA clinical trials are underway and for the first time, FARA is sponsoring one of them. More than 100 scientists and FA advocates from all over the world attended our mid-year therapeutics symposium and made presentations on exciting new therapies. We received more qualified grant requests than ever before, we have the most robust research portfolio we have ever had, and FARA researchers are making exciting headway. This year, alone, seven additional pharmaceutical companies have contacted FARA to express specific interest in our work and to explore new collaboration.*

*Looking ahead to 2010 and beyond, FARA faces a new exciting path to treatments and a cure. There is tremendous progress being made and growth in the scientific community's commitment to our goal. New potential therapies continue to surface and new clinical trials continue to take shape, spurred on by FARA's collaborative culture. But, with this growth of commitment and opportunity comes a heightened need for funding.*

*You may be wondering, "why do we need more now?" During FARA's first ten years, the majority of the research grants were awarded for basic, discovery research (understanding the cause/mechanism of the disease). Our typical grant amount was \$25,000-\$75,000. Over the past three years, we have seen change in three key areas: 1) the number of grant requests has increased; 2) the amount of money requested has increased, and 3) the type of research being proposed has changed. Nearly all of the research proposals now focus on translating and developing basic research discoveries into treatments or on clinical (human) research. Typical grant amounts now range from \$100,000 to several times that amount.*

*A comparison of grant activity shows that in 1999, FARA awarded two grants totalling \$29,000, while by the end of 2009, FARA will have reviewed more than 40 grant requests and made more than 20 grant awards approaching \$2 million in funding.*

*In order for FARA to continue to support all of the most promising research — including research that advances potential treatments from the laboratory to patients —*

*and to maintain our urgent pace, we must significantly increase our funding. Therefore, I would like to ask each member of the FA community to help us reach our goal, within the next three years, of \$5 million annually in FARA-funded research. That is our estimate of how much will be required to cross the finish line together. FARA does not have a capital campaign or an endowment fund. This first-ever annual appeal is to inform you, our supporters, of the resources required to meet our goal. This goal is achievable only with your help.*

*We recognize that we are in challenging economic times and that many of you are already donors or fundraisers. Without the generous support and leadership of FA families and friends like you, we would not be at this juncture. But, we also know how important it is to you to understand FARA's needs and how you can help.*

*Your gift will have a real impact. Whether you choose to increase your personal donation, raise more funds, or move from an annual donation to a monthly giving program, you will be helping FARA take FA research to the next level. With greater funding, we will sponsor more research, enhance our network of clinics, move more treatments from the lab to patients and educate more people about FA.*

*On a personal note, if you are not already doing so, you may wish to consider a monthly giving program that is directly deducted from your credit card. I intend to take this step with my personal giving. It provides the opportunity to spread out your gift amount allowing you to consider a larger gift while providing you with better control over your monthly expenses. It also will provide FARA with more even cash flow for grant allocation. If you wish to contribute online, visit our website at [www.cureFA.org](http://www.cureFA.org).*

*Thank you all for helping to bring FARA to this pivotal place and time in our research.*

*Together, we will eliminate the devastation of Friedreich's Ataxia.*

*Warmest regards,  
Ron*

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

**2009 Fundraising and Development Initiatives**

**Added a Chief Development Officer, Juliann Green –**

After an exhaustive search, FARA filled a new position that focuses on growing FARA's fundraising capacity and securing larger institutional and individual donors.

**Created Volunteer Development Advisory Board –**

FARA formed a new volunteer board of business professionals to help build awareness for FARA and FA and to grow FARA's support network. This newly formed team is a critical component to identifying new donors and important corporate supporters, along with increasing awareness for FARA and FA in the media.

**Enhanced technology infrastructure for donor development and fundraising –**

In an effort to better manage donor relations and support existing fundraising, FARA instituted a comprehensive donor database, added online fundraising pages for events, and purchased event/auction management software. This infrastructure enables FARA to establish and maintain stronger donor relations, better donor communications, support increased fundraising activity, and maintain IRS reporting compliance.

**Established and executed FARA's first branded event –**

We have been fortunate to have active, committed and successful volunteers around the country to organize and execute annual events on behalf of FARA. It is because of this volunteer network and success that FARA was advised to establish a branded event. The goal of the branded event is to gain a presence on the national fundraising stage and increase fundraising dollars. FARA is fortunate to have collaborated with Kyle Bryant over the past three years on such an event – Ride Ataxia – and we are proud to announce Ride Ataxia as FARA's branded event. Ride Ataxia Philly, held Oct 25, 2009, was the first highly successful event of this new partnership. Three rides are already being planned for 2010.

**Selected Kyle Bryant as Director of Ride Ataxia and**

**FARA's official spokesperson –** Kyle and Ride Ataxia enable FARA to develop a much-needed face and story to help build awareness in the media. Kyle will spread the FARA message to the research and medical communities and the general public through public speaking engagements, medical education opportunities and through his already successful Internet presence.

**What does this mean for FARA's budget?**

In 2008, FARA's work was executed by a staff of three. Fundraising and operational expenses were slightly less than 10 percent of our income, which means that

90 percent of our funds went to research and related programs. In 2009, FARA has five full-time and two part-time staff. Our expenses have increased but so has our income as we have supported nearly four times the number of fundraising events around the country, and awarded nearly \$2 million in research grants. While all the numbers are not yet in for this year, FARA's fundraising and operational expenses will be well below the 25 percent benchmark for the non-profit industry. We are confident that the investment we have made in staff and infrastructure this year will result in a significant increase in dollars for FA research in 2010 and beyond.

We realize that communication and transparency are incredibly important. We are committed to communicating our progress and other information to you in convenient and efficient ways, such as this letter and an annual report. We encourage you to frequent the FARA website where content is continually updated. Our financial information is located there along with information on current research.

Please don't hesitate to contact me or any member of the FARA team if you have specific questions, concerns, or feedback on FARA's organizational growth and direction. It is only with our combined efforts that we can achieve our mission to Cure FA. ■

## Holly's Hope- Jewelry For A Cure

Affordable handmade bracelets, lanyards,  
eyeglass holders, earrings

<http://hollyshope.weebly.com/jewelry.html>

ALL proceeds from these sales will be donated  
to FARA to find a cure. To order please contact us at

[hollys.hope@comcast.net](mailto:hollys.hope@comcast.net)



## Ride Ataxia Joins FARA

By Kyle Bryant



Dear FARA Supporter,

Ride Ataxia began three years ago with one pedal stroke. My first trike ride was seven miles. Since then, Ride Ataxia has traveled 3,300 miles, involved over 500 participants, and funded six research grants for a total of more than \$800,000 for FA research. This effort has included a 60-day 2,500-mile trip from San Diego, CA to Memphis, TN; a 13-day, 650-mile trip from Sacramento, CA to Las Vegas, NV; and a 4-day, 200-mile trip from Portland, OR to Seattle, WA.

As Ride Ataxia has grown by leaps and bounds over the past three years, we have addressed the need to share the adventure with people of all abilities, largely by changing the format of the Ride to include shorter weekend rides in which all can participate. Throughout this three-year process, Ride Ataxia and FARA have recognized significant benefits by working together directly. As many of you know, I was thrilled to join FARA in October as a full-time employee, fueled by the knowledge that the organization and the Ride would mutually benefit from the others' strong foundation, and that our combined forces will bring us closer to achieving our shared goal: to cure FA.

FARA's first Ride Ataxia event took place on October 25, 2009 in Philadelphia. With the strong support of local Outback Steakhouse restaurants and their business partners, Ride Ataxia Philadelphia involved 350 participants and raised an astonishing \$120,000 for FA research. The participants included eight people from the FA community on trikes, and representatives from 20 families affected by FA – some local, and some traveling up to six hours to participate! Because we deal with such a rare disease, these numbers mean a great deal to us.

Next year Ride Ataxia will build on the momentum of our resounding successes in Philly and sponsor three challenging, yet family-friendly bike rides: in Sacramento, CA. San Diego, CA, and once again in Philadelphia, PA. We plan to expand further in the coming years.

As we look to the future, I invite you to join us in this adventure with participation and support of Ride Ataxia events in your area. With your continued support we will continue to turn the crank towards a cure for FA!

See you soon,

Kyle Bryant  
Ride Ataxia Founder and Director

## Collaboration and Fundraising Efforts Help to Expand FARA Grant Program

By Jennifer Farmer

As the pace of FA research has gained momentum and the focus has evolved from understanding the core mechanisms of the disease to discovery and advancement of potential treatments, FARA's grant program and research portfolio has grown to meet these increasing needs. Also affecting the demand for funding are the US and global economic challenges that have impacted both academic and industry researchers, many of whom are reporting more difficulty obtaining grant funding through traditional sources.

As of October 2009, FARA has received a record number of grant requests (>40!) that exceed \$4 million in grant dollars solicited during this year alone. All FARA grants undergo a competitive external/peer review and internal review, which guides all funding decisions. We have already made more than 20 grant awards and anticipate exceeding \$2 million in direct grant support before the end of the year – our largest contribution ever to research.

With such a great need for research funding and resources, FARA would like to recognize and thank our many funding and advocacy partners, including the Muscular Dystrophy Association, FARA-Australasia, the National Ataxia Foundation, the American Heart Association, Go-FAR, Ataxia UK, FEDAES and FA-Babel Family, as well as government agencies such as the U.S. National Institutes of Health. FARA realized from the beginning that treating and curing FA was far too great a challenge for any one organization and that all such partners would need to put their shoulders to the same wheel in active collaboration. This collaborative approach has worked extremely well, ensuring that our efforts are complementary and additive, not duplicative, getting us all to treatments more quickly together than alone.

### **FARA's Grant Awards for 2009 are working to advance research in the following ways:**

- *Discovering novel drugs that improve mitochondrial function, increase frataxin protein levels, or increase expression of the FA gene*
- *Improving and expanding the cellular models which researchers use for evaluating new therapeutic targets; specifically, utilizing advances in stem cell technologies to create neuron and cardiac cell models directly from FA patient skin cells*
- *Supporting the International Collaborative Clinical Research Network in FA which focuses on clinical research including: developing and validating clinical outcome measures and biomarkers that are necessary for clinical trials, capturing the natural history of FA, building a DNA bank for genetic modifier studies, and facilitating the implementation and delivery of clinical trials*
- *Accelerating the pace of bringing new discoveries to clinical trials; including funding a clinical trial of Chantix*
- *Exploring and testing new hypotheses that further inform and expand our understanding of FA at the genetic, protein and cellular levels*

- Supporting young/new researchers with innovative ideas and a commitment to FA research
- Reducing the morbidity and mortality caused by cardiac disease in FA

For a complete listing of grants funded for 2009, please visit the FARA website at <http://www.curefa.org/grants-awarded.html>

## Spotlight on the Kyle Bryant Translational Research Awards

This year, FARA and the National Ataxia Foundation (NAF) were pleased to make three grant awards through the Kyle Bryant Translational Research Award Program. The Kyle Bryant Translational Research Award is significant because it demonstrates the full collaboration and participation that is essential from the patient, research, and advocacy communities to treat Friedreich's ataxia. We are most grateful to all of the participants and supporters of Ride Ataxia who raised funds and to all of the researchers who submitted applications for this award.

**This year's Award winners are:**

### Protein Replacement

**Principal Investigator: Dr. Mark Payne**  
**Indiana University School of Medicine**

#### **Optimizing delivery of frataxin using cell penetrant peptides**

Dr. Payne's Lay Summary — The Payne laboratory has been working on a therapeutic approach toward frataxin protein replacement. We have recently shown that we can deliver the missing protein that causes FA, frataxin, into the mouse to rescue the mouse model of this disease. These exciting findings are being prepared for publication, and we have now focused our laboratory on developing protein replacement strategy as a new drug to treat Friedreich's ataxia. In order to move this research into preclinical development as a drug, we first need to determine the best molecule to move forward and optimize the conditions for expression and purification of the protein we are developing, called TAT-Frataxin. Finally, a key tool in this research that is missing is a good antibody that will allow us to detect frataxin expression in mouse tissues. This project will solve these 3 problems within the year of this research to enable preclinical development of TAT-Frataxin as a drug for Friedreich's ataxia.

### Drug discovery toward improving mitochondrial function

**Principal Investigator: Dr. Gino Cortopassi**  
**University of California, Davis**

#### **Screening for mitofunctional Friedreich's ataxia therapeutics**

Dr. Cortopassi's Lay Summary — Mutations in the frataxin gene result in defects in mitochondrial function that are the likeliest cause of neuro- and cardiodegeneration in Friedreich's ataxia. The Cortopassi laboratory has developed an assay with which to screen thousands of compounds for their positive effect on mitochondrial function, based on mitochondrial Oxygen (O<sub>2</sub>) consumption. A cell line in which frataxin is inhibited shows that mitochondrial O<sub>2</sub> consumption is decreased. In addition, other cell models of mitochondrial disease, i.e. cells bearing mitochondrial mutations, also exhibit decreased mitochondrial O<sub>2</sub> consumption.

The goal of the proposed research is to screen large drug libraries to identify drugs that increase mitochondrial O<sub>2</sub> consumption and rescue mitochondrial deficits that are the specific consequence of deficits in frataxin. These drug leads might serve as the progenitors of effective small-molecule therapy for Friedreich's ataxia.

### New strategies to regulate FRDA gene activity

**Principal Investigator: Marek Napierala**  
**University of Texas, MD Anderson Cancer Center**  
**Crosstalk between microRNAs and iron metabolism in pathogenesis of Friedreich's ataxia**

Dr. Napierala's Lay Summary — Deficiency of frataxin leads to significant changes in the genetic program of affected cells — many genes become more active and some others become less active. This is also the case for a group of small molecules — termed microRNAs — that regulate the activity of other genes. We will study the changes in the activity of microRNAs in FRDA to see how abnormalities affect the activity of genes which are responsible for the localization and amount of iron in neuronal cells of patients with Friedreich's ataxia. This work also aims to uncover the pattern of detectable microRNA molecules that is specific to FRDA. This "microRNA signature" characteristic for FRDA will help to evaluate the effectiveness of therapeutic approaches aimed to increase activity of the frataxin gene or intended to regulate distribution of iron in neurons of FRDA patients. Misregulated microRNAs as well as genes under their control may also become new targets for therapeutic intervention in FRDA. ■

### Attn: FA Scientists — 2010 grant submissions

FARA accepts grants 24/7/365 to be considered for general funding and in addition we invite applications against several named awards.

**New Investigator Award** – applications due March 1, 2010 and September 1, 2010

**The Bronya J. Keats Award for International Collaboration in Research on Friedreich's Ataxia** – applications due June 1, 2010

**Kyle Bryant Translational Research Award** – application deadline TBA

### Special Announcement

**AHA/FARA Friedreich's Ataxia Cardiology Research Award** – applications due in January 2010. Applications must be submitted to AHA — <http://www.americanheart.org/presenter.jhtml?identifier=3041239>

We strongly encourage all researchers considering submitting any type of proposal to submit a letter of intent via email. All grant proposals must be received through FARA's online grant management system. <http://www.curefa.org/RPMP>

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

### Dr. Mark Payne

Interviewed by Paul Marcotte



*Dr. Mark Payne of Indiana University School of Medicine is one of the world's leading Friedreich's ataxia researchers.*

*He is a member of FARA's Scientific Advisory Board and is currently developing TAT-Frataxin as a possible therapy for Friedreich's ataxia. FARA announced in October that Dr. Payne was one of the investigators to receive a \$120,000 Kyle Bryant Translational Research Award for research into "Optimizing delivery of frataxin using cell penetrant peptides." Dr. Payne's research explores approaches to frataxin protein replacement in order to determine the best molecule for TAT-Frataxin to move forward into preclinical development. In 2008, Dr. Payne was the first recipient of the joint American Heart Association/FARA Friedreich's ataxia Award.*

#### **Can you tell us a bit about your current research into TAT-Frataxin. What is TAT-Frataxin?**

TAT-Frataxin is a protein consisting of normal human frataxin attached to a short protein called TAT. This type of artificially created protein is called a "fusion protein" and means that the genes encoding two separate proteins have been attached to each other to make a single protein, TAT-Frataxin in this case. The TAT protein is a short peptide that has the ability to move across both cell (cell penetrant) and mitochondrial membranes and pull a larger protein with it inside the cell. So, as a fusion protein, TAT-Frataxin has two functions: the TAT pulls the normal human frataxin protein into the cell and mitochondria, and the frataxin is recognized by the mitochondria and functions as normal frataxin protein.

#### **Why is Frataxin important in FA?**

Frataxin is the protein that is missing in Friedreich's ataxia. The gene encoding this protein is defective and thus, no frataxin is produced or else it is produced in such low amounts that it can't do its job effectively. Studies have shown that if the cell ever has frataxin, it will handle this protein appropriately and has normal function. Frataxin is an iron binding protein that functions inside of mitochondria to bind and present iron to other protein complexes. In its absence, these other critical enzymes and proteins cannot function effectively. As a result, the mitochondria eventually stop producing energy and the cell no longer functions normally. Loss of mitochondrial function is critical for tissues, such as brain and heart, which use lots of energy.

#### **Your early results have been promising. What has your research into TAT-Frataxin shown so far?**

We are excited about our findings. To date, we have been able to show that a TAT-Frataxin protein will move across the cell membrane into mitochondria. For cells in culture from patients with FA, we've been able to show that treating these cells with TAT-Frataxin restores their resistance to an oxidant stress from iron exposure. For the Friedreich's ataxia mouse model from Helene Puccio, we've been able to partially rescue the animals with TAT-Frataxin and markedly extend their lifespan. They have better heart function. This is very important because these animals represent the most severe phenotype of FA in that they are completely lacking frataxin expression in certain tissues. These findings have encouraged us to pursue TAT-Frataxin to see if we can optimize its performance and function.

#### **What is your goal with your TAT-Frataxin research?**

There are two goals for this research: 1) Develop a therapy for Friedreich's ataxia (FA). We are hopeful that we can develop TAT-Frataxin, or a variant of this protein, to function effectively as a new drug to treat patients with FA. 2) Take the lessons we learn from working with TAT-Frataxin and expand this technology to other mitochondrial diseases. This will help us develop new drugs and therapies for other diseases, and discover new knowledge about mitochondria.

#### **Your research has been funded by Shire Human Genetics, Inc. Is this research a priority for the company?**

Yes, they are deeply committed to this approach and this is a priority for this company. They have a mission to develop therapies and cures for rare and difficult diseases and have been successful in the past.

#### **What is the timeline before TAT-Frataxin could be in patient clinical trials?**

This is hard to guess at because there are so many variables and experiments that have yet to be done. If results from additional animal and laboratory studies progress as hoped, then it is quite possible that a TAT-Frataxin molecule could move into pre-clinical studies within two years. Phase I studies would follow after these were completed and had been approved by the FDA. At the earliest, five years would be realistic for significant clinical trials.

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Heineken and Outback present FARA with a generous contribution of Heineken with a Heart proceeds in Baltimore, MD.

## OSI Restaurant Partners, LLC Named FARA 2009 Partner of the Year

*FARA recognizes that collaboration and partnership are absolutely essential for success. The challenges that we face in accomplishing our mission require that we establish partnerships with individuals and organizations from government, industry and other advocacy groups. We believe in recognizing and celebrating these partnerships and the people who make them successful.*

This year FARA presents our 2009 Partner of the Year Award to OSI Restaurant Partners. The presentation was made to the OSI leadership, Bob Basham, Trudy Cooper, Bill Allen, Jody Bilney, Joe Kadow and Concept Presidents in October at the retirement event of Paul Avery, Chief Operating Officer of OSI and member of the FARA Board of Directors. Paul and the leadership team at OSI have met and exceeded the criteria for FARA Partner of the year (see sidebar).

OSI is a distinctive organization whose leadership and employees ALL demonstrate and live supporting community through outstanding generosity and hospitality. The leadership and employees of the OSI concepts, Outback Steakhouse, Carrabba's Italian Grill, Bonefish Grill, Roy's and Fleming's, have supported the FARA mission through fundraising, in-kind contributions, raising awareness, creating community support for our families, and being wonderful and supportive partners and friends. They have invited FARA into their family and introduced us to a network of colleagues and friends that has also embraced our mission.

The results of OSI's efforts within the past 12 months include generating more than \$750,000 in contributions to FARA.

While the efforts of OSI are too numerous to mention in total, we will share a few statistics here to give you an idea of the astonishing way that this organization has transformed FARA fundraising in 2009:

- More than 100 luncheons that promote community support and fundraising with FA families and their friends
- Presenting sponsorship of the inaugural 2009 FARA Energy Ball
- Presenting sponsorship, personal participation, fundraising and media support for Ride Ataxia events (Ride Ataxia III - raising >\$120,000 - and Ride Ataxia Philly - raising \$120,000) all of which have substantially helped FARA and Ride Ataxia move forward in 2010 to a national branded event
- Hosting larger fundraising events such as the Chesapeake Bay Clay Shoot and the 2009 WE Cup golf event and dinner (the culmination of a year of fundraising by the Carolinas restaurants)
- Support for numerous FARA family fundraisers including dinners, walks and marathons
- Participation and organization of numerous employee/company fundraising events
- Making FARA the beneficiary of many restaurant programs and promotions
- Donation of time from restaurant managers and employees at every level of the organization to organize and support these events, and serve the guests
- Raising awareness through increasing media opportunities and marketing support

The FARA community is extremely grateful for OSI's leadership, generosity and friendship. ■



Heineken with a Heart in Las Vegas, NV.

## 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

**You have taken a leading role in pulling together researchers to have a better understanding of various heart problems in FA patients. What specifically have researchers been doing recently?**

Initially, clinicians logically focused on the neurologic impact of FA because this was the most prominent physical finding. However, the heart is affected as well and is frequently a cause of mortality in patients with FA. Researchers are now beginning to look at mechanisms for why and how the heart is affected in this disease, and how it can be helped. For example, the heart in patients with FA frequently becomes very thick and doesn't function normally. This leads to problems later when surgeries are required, and can cause dangerous arrhythmias. Researchers are now looking at ways to decrease the thickness of the heart and improve its function. However, we still need more clinical studies of the heart in this disease because of the variability in how FA can affect the heart. More research on heart function using animal models of FA is also needed to determine the mechanisms of heart damage in this disease. In particular, better animal models are needed that more accurately mimic the genetics of the human FA patients.

**At some recent scientific meetings, researchers have indicated that some earlier assumptions about how FA impacts the heart have been incorrect? What was wrong, and what is the current thinking?**

In some patients with FA, the heart becomes very thick. Although the heart still 'squeezes' well and can pump the blood effectively, the increased thickness of the heart means it can't fill with blood as easily. If this happens, it makes it much harder for the heart to adjust its output to match the demand for more blood to be pumped, i.e., if the blood can't get into the heart, then there isn't as much blood for the heart to pump out. It also means that the heart won't tolerate large changes in blood volumes, such as during surgery for scoliosis, and can fail because of this stress. Greater education of physicians and families about the nature of this cardiac dysfunction in FA is needed, as are more clinical studies of the impact of FA on the heart.

**How is the American Heart Association working with FARA and FA families on these issues?**

The AHA has helped fund studies on heart function in animal models of FA and in patients. This is a significant recognition by a large and definitive scientific group that the heart is a key organ affected in FA and needs greater study.

**What new insights do you expect from the current research into heart function in FA patients?**

Hopefully, we will determine three things: 1) The mechanism for heart dysfunction in this disease. 2) How we can improve heart function in this disease to improve lifespan and decrease mortality. 3) Why some hearts are affected and others are not. This is a very puzzling aspect of FA and may offer clues about gene expression that can be taken advantage of to develop new therapies.

**As you consider other potential FA therapies, what do you see as the most promising research underway?**

At this time, two approaches would seem to offer the greatest possibility as a therapy for FA: 1) Those therapies designed to increase expression of native frataxin. These are primarily the HDAC inhibitors, which help increase expression of the normal frataxin protein from the defective FA gene. Significant work is well underway to develop small molecules that can increase gene expression without serious side effects. 2) Those therapies designed to increase the amount of frataxin protein in the cell. This is primarily by protein replacement (or enzyme replacement) strategies, such as TAT-Frataxin. Once again, work is well underway to develop cell penetrant fusion proteins, like TAT-Frataxin, that can deliver the missing frataxin protein to the mitochondria in all tissues. Both approaches, HDAC inhibitors and protein replacement therapy, come at the problem of FA from different directions and have unique strengths and weaknesses. Both approaches also have the strong appeal of potentially offering new technology to cure other diseases as well. Finally, it is quite probable that a combination of both approaches, which takes advantage of their combined strengths, will provide a cure for FA.

**How long do you think it will be before there is a cure for FA?**

Hard to guess on this but work is moving very, very fast. This is largely due to the high degree of focus and organization of groups such as FARA (Friedreich's Ataxia Research Alliance). In particular, FARA has advanced our scientific understanding of FA while keeping attention focused on a cure based on this understanding. Again, I am hoping for clinical trials of both approaches above, and a therapy to advance to market within seven years. ■

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

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



Guests clad in Hawaiian attire enjoy the camaraderie, surrounded by balloons for the upcoming "Balloon pop" at the FAI-TH event.

balloon pop add to the festivities and success of the Rupel family's annual event. At a certain point in the evening, the attendees pop the helium balloons they've bought to discover the gift they've won — perhaps a trinket...or an iPod!

Just what does FA-ITH stand for? "Friedreich's Ataxia In The Heart" has a three-fold meaning, Bart says. First, the early years of the FA-ITH event were held around Valentine's Day; second, the scariest thing about Friedreich's ataxia is that it affects the heart; and third, the Rupels and their guests have faith that there will be an effective treatment and a cure for Friedreich's ataxia.

Two hundred faithful people came June 20 to raise money for FA research in honor of Bart and Brenda's 19-year-old son, Matt, who has FA. The Santa Clara community's incredible participation reflects how involved the Rupels have been in their community. They give and others want to give back. Families of school friends are particularly helpful on the committee and each member has found their niche. The toughest job, says Bart, is ASKING — and he feels blessed with friends who have that strength. The community's response is evident when one sees the overflowing silent auction table.

Outback's incredible food makes it very easy for the Rupels' committee, who don't have to concern themselves with feeding the crowd! Thanks once again to the Outback team led by the awesome Pat Kruk.

How do the Rupels keep expenses low? In working toward that goal, a close friend makes all the beer and even auctions off beer-making lessons. Bart strongly suggests not charging guests for beer and wine but leaving out a BIG tip jar! Of note: a delicious tropical drink is also a draw, in line with the Hawaiian theme that the Rupels have

adopted. Also finding a reasonably priced venue is of utmost importance and Bart says to be sure to book early.

Local media connections have resulted in some personal stories of Matt's struggles and strengths in dealing with FA, bringing greater awareness to Friedreich's ataxia. Matt's personal story is also presented at the FA-ITH event and this reminds the guests just why they're there. As part of the festivities, special thanks and plaques are given to each of the very generous workers to assure that everyone feels appreciated.

Having planned four FA-ITH events to date, the Rupel family has faith that they have inspired other families dealing with FA to "step up to the plate" to work with their own communities, having fun while raising money for FA research. ■

A lucky guest with arms loaded, departing from the Santa Clara FA-ITH event is greeted by a big grin from worker Paul Konanz.



## MEMORIALS

From January 2009 to October 2009, FARA has received over \$20,000 in memory of the following individuals:

John Andresen, Helen Barnett, Thomas Barnett, Michael Breedlove, Wilma Brock, Dick Buchanan, Arnold Bucklow, Deborah Cappicille, Mel & Jim Caruso, Joshua Chalcraft, Virginia Cooper, Selma DeRosa, Jan DeSaw, Flavio and Barbara DiBonis, Mildred Evans, Brenda Farrington, Ruth Kermani, Marilyn Fanelli Lumio, Gertie Gray, Mrs. Orell Gregg, Christopher Goddard, Paula Bikhazi-Hawi, Marcelino Huerta III, John Maxwell Jacquin, Gabrielle Johnson, Walter H. Jones, Joseph Kent, Francis Leonard, Dave Lewis, William Londergan, AnnMarie Lowe, Milton Marino, Ginny Martinau, Anthony Minei Sr, Nathan Nickerson, Marie O'Brien, Kathleen Paulsen, Angelo Pepe, Elena Raymond, Walter Riggs, "Rosie" Rosencranz, Marilee Skinner, Jenifer Sullivan, Charles Testa, Vicki Wall, Gladys Chan Lee Wan Ying,

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 Grows and Advances the FA Treatment Pipeline

By Jennifer Farmer

Our FA Treatment Pipeline has been a critical tool for FARA to visually communicate the progress of “lead candidates” for potential treatments. Along the horizontal axis, lead candidates are grouped based on the mechanism of action or approach to treatment, i.e., how it might work or the problem it addresses. The vertical axis indicates the stage of the research, or where it is in development. The first two stages, research and pre-clinical, take place in the research laboratory. The stages Phase 1 on are phases of clinical trials, or studies in individuals with FA. The hope for any of the lead candidates is that they will climb to the top and become approved treatment for FA; however, not all lead candidates will progress from the bottom of the chart to the top.

### Support development of better models

While we are very optimistic about all of the lead candidates in the FA treatment pipeline, we know not all proceed to approved treatments for FA. Based on research results, it is possible that a candidate that looked good in laboratory studies does not show the same promise in humans. In such a situation after a Phase 1, 2, or 3 study, the candidate would be taken off the chart. One of FARA’s research strategies is to support the development of research tools such as cell and animal models that can be used during the early stages of evaluation of a lead candidate to help ensure that candidates that progress from pre-clinical to human studies have a higher likelihood of success. This is important because clinical trials have greater cost and risk and it is best to identify candidates destined to fail as early as possible.

### Keep the pipeline full

Another research strategy is to keep our pipeline full of candidates; the more shots on goal the better. New candidates appear on the pipeline when we have sufficient research data to suggest a benefit to FA patients or when a specific approach to treatment has been defined and can be evaluated in specific cell and animal models. For some candidates, the bar will be at the base of the chart in the research phase – this indicates that this candidate has emerged through laboratory research — drug discovery. Other candidates may first appear with the bar indicating it is already in clinical trials. Candidates that appear at the stage of clinical trials are most often drugs that have already been discovered and investigated and in some cases approved for treatment of other conditions. For example, we just added a new candidate to the pipeline called “Protective cytokines/EPO-like compounds” and it is at the Phase II stage with clinical trials starting now. The research and pre-clinical work was performed initially in other disease models. Based on recent studies suggesting that EPO might be beneficial in FA, the investigators quickly translated this data to FA and proposed a clinical evaluation approach versus a laboratory-based approach.

### Progress in the FA treatment pipeline

In 2009 there has been significant progress in the treatment pipeline. There were seven clinical trials initiated or ongoing this year and we are anxiously awaiting the results.

### Three new candidates in clinical trials

- *EGB76, a ginkgo biloba-like compound, is in a Phase II trial being conducted by Ipsen in France. An EPO-like compound that could have neuro-protective benefit as well as potential to increase frataxin is in an early Phase II study being conducted by Lundbeck in various European FA centers.*
- *Chantix, a drug approved for smoking cessation, improved balance symptoms in various ataxia patients and is now in a Phase II clinical trial in two sites in the US. Of note, this trial has been funded by FARA.*
- *Pioglitazone, a drug approved for the treatment of diabetes, is being evaluated in a phase II clinical trial in France to see if it improves neurological symptoms in FA patients.*

### Completion of clinical studies

- *A Phase III study of Idebenone / Catena® was completed in the U.S. in early 2009, however an extension study is ongoing and a European Phase III study is reaching conclusion around the end of the year. While the data from the US Phase III study did not demonstrate benefit at the level of statistical significance, the company is hopeful that data from the U.S. extension study combined with data from the European phase III Catena® trial will achieve statistical significance so the drug can be submitted for approval as a treatment for FA.*
- *An international Phase I/II study of Deferiprone (iron chelator) was completed in July/August 2009. We are all anxiously awaiting the results of this study which are anticipated before the end of the year.*
- *A Phase I study of A0001 was completed earlier this year and results were presented at the FA therapeutics symposium in July. This study evaluated safety and dosage. Plans for a phase II study of A0001 were announced and such a study is anticipated to begin before the end of the year.*

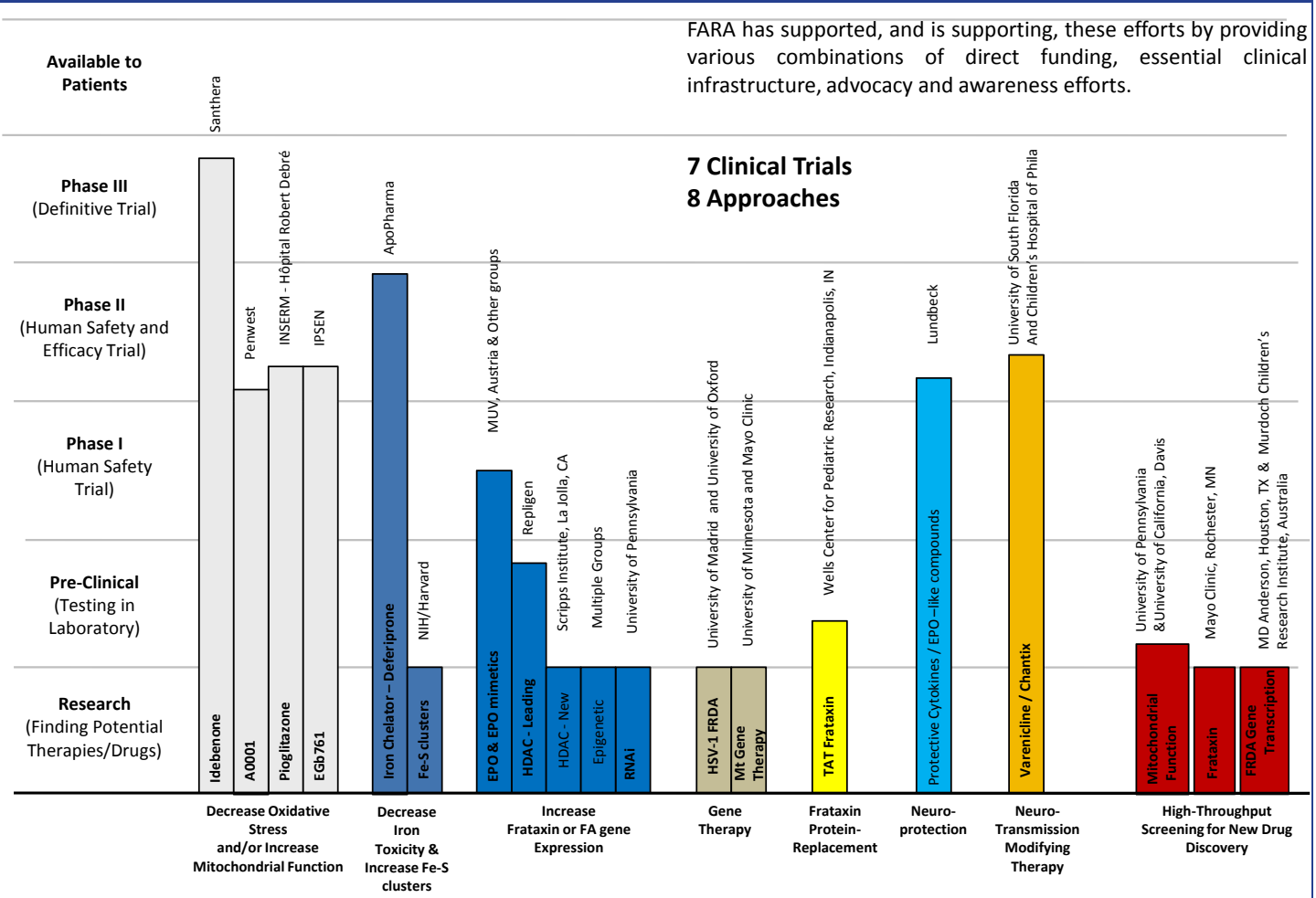
The FA treatment pipeline is updated on a regular basis on the FARA website. There is also a more detailed description of each of the lead candidates and the status.

<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](http://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. ■

## Friedreich's Ataxia Treatment Pipeline

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



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## FARA Organizes First FA Therapeutics Symposium

By Ron Bartek and Jennifer Farmer

Early in 2009, FARA recognized the need to organize a symposium around the most promising emerging therapeutic developments. We envisioned a meeting of 30-40 participants, but by the time we heard back from all the scientists and partners that “just had to be there,” we had three times that number and sponsorship support from both industry and advocates. What a wonderful tribute to the fact that with all of us working so hard together to support the best FA science in the world, we have been successful in growing the field tremendously, increasing excitement among scientists, and heightening researchers’ unquestionable commitment to curing FA within the shortest time possible.

More than 100 FA researchers and our advocacy partners from around the world gathered July 15-17, 2009 for the FA Therapeutics Symposium in Philadelphia, PA. Presentations and discussions highlighted:

- *progress in the development of previously identified therapeutic candidates, such as HDACI and TAT-Frataxin results from clinical trials including the Phase I study of A0001 and Phase III of Idebenone*
- *recent discoveries that point to new therapies*
- *advancements in new cell models and drug discovery and development assays*
- *clinical research including biomarker studies and new clinical outcome measures.*

In addition to sharing progress and insights on various aspects of FA therapeutics and development, participants were encouraged to discuss and identify a number of key assets that would accelerate progress significantly. By defining specific resources and research that are needed to more efficiently and effectively move drug candidates forward from the research lab to patients, we are able to concentrate our efforts to develop these assets. Since the symposium, FARA has awarded research grants, formed task forces, and fostered collaborations and new partnerships to begin to fill these gaps.

### Key Assets Needed to Accelerate Progress

**Human FA Neuronal and Cardiac Cell Models** – To date, the human FA cells used to test potential therapies have been those which are easily obtained and maintained in the laboratory: blood cells (lymphoblasts) and skin cells (fibroblasts). Because FA is a neuromuscular disorder, testing in blood and skin cells is not optimal. This asset was considered so important and urgent that the international investigators working separately on this project agreed to meet in California immediately following the Symposium to collaborate on a faster, more efficient way to achieve this goal.

### Detailed Characterization and Optimization of FA Mouse

**Models** – Animal models of disease are powerful research tools. Several different FA mouse models are currently being used in early stages of drug screening and in later stages of drug development to help determine which molecule in a series of molecules might be the best candidate to take into a clinical trial. But these models have some shortcomings, such as they do not have strong phenotypes (do not manifest significant physical symptoms) and are not well-characterized (the cellular changes that result from their FA mutations are not well-defined). Consequently, FARA has formed an animal model task force consisting of the investigators who developed the current models, a representative of The Jackson Laboratory, and FARA representatives. The Jackson Laboratory, the world’s premier animal model facility, received a FARA grant to attempt to improve current mouse models and breed them for distribution to labs for FA research.

**Additional Assays for High-Throughput Screening** – Developing FA assays that are useful in what is called “high-throughput screening” allows for hundreds of thousands of drug compounds to be tested rapidly. Thus far in FA, one such assay, supported by FARA and NIH grants, has been tested in the NIH high-throughput screening system. This first FA approach involved a yeast FA assay well suited to identifying compounds that might improve mitochondrial function in FA. The high-throughput screening of hundreds of thousands of compounds against this assay resulted in promising “hits” that are being advanced by the scientist that developed the assay. Symposium participants pointed to the value of developing assays that would enable such screening for compounds that might, for example, increase frataxin protein expression or otherwise increase frataxin protein levels.

**Refined Biological Measuring Tools** – The FA community continues to need better and better tools to measure key biological factors in FA (e.g., frataxin protein levels, cellular energy production, cellular oxygen use, oxidative damage) as well as functional measures (e.g., tests that demonstrate a physical change or outcome that can be linked to FA and answer the question: Is the individual with FA better, worse or the same?).

### Importance of Collaboration in Accelerating Progress

As the fourth FARA-organized FA international scientific conference, the Symposium was a remarkable manifestation of the high level of collaboration that continues to grow among all participants and that will be instrumental in getting us to treatments and a cure together. When leading researchers, drug companies and advocacy organizations from all over the world come together to share data, insights and plans openly and to learn from others in the field, they accelerate progress for all. ■

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

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



## FARA and USF Welcome 600 to Energy Ball

By Felicia DeRosa

On August 29, 2009, the first annual FARA Energy Ball, benefiting the Friedreich's Ataxia Research Alliance and the University of South Florida, welcomed 600 guests to three days of high energy activities true to its namesake. The event kicked off with an educational symposium, included tennis and golf tournaments, and culminated in a gala dinner/auction. Hosted by a dynamic and creative organizing committee in Tampa, each of the event activities brought complementary interpretations of the theme "energy for the cure."

The "Understanding Energy for the Cure" symposium speakers educated supporters on Friedreich's ataxia, the status of FA research, and the impact of living with a progressive neuromuscular and energy deprivation disease. Supporters showed their energy for and commitment to the cause by overfilling the designated space during talks from USF researchers, representatives from FARA, and members of the patient community. The next two days were busy with a tennis and golf tournament appealing to the active energy of the sports enthusiasts in attendance.

Guests of the weekend's culminating event, the FARA Energy Ball, were greeted at the vibrantly decorated À la Carte Event Pavilion. The artistic vision of the event included variations on the event logo, including centerpiece flower arrangements in the shape and color of the energy ball. During the cocktail hour, guests enjoyed activities that creatively incorporated themes of energy and balance such as an oxygen bar and traveling balance ball acrobats. The evening included a deliciously catered sit-down dinner, competitive live and silent auctions, and entertainment by Las Vegas entertainer, Gordy Brown.

Throughout the event-planning process, an impressive amount of in-kind services, donations of auction items, and volunteers

pitching in to help kept expenses low and contributed to make this event one of Tampa's best fundraisers. In its inaugural year, the event raised in excess of \$900,000 as well as the energy of all in attendance. We are excited to announce the dates for the 2010 FARA Energy Ball which is scheduled for August 26 - 28. A special thank you to the many sponsors whose generosity made this event so successful:

**Presenting Sponsors-** OSI Restaurant Partners, LLC; Proximo; MillerCoors.

**Platinum Sponsors-** Paul & Suzanne Avery; Diageo North America; Foster's Wine Estates

**Gold Sponsors-** Anheuser-Busch, Inc; Beam Global Spirits & Wine; Chris T Sullivan Foundation; Coca Cola North America; CORE; Danker Basham Foundation; Heineken; Paul & Mary Jacobs; Kraft Foodservice; Pernod Ricard USA; PFG Performance Food Group; Southern Wine & Spirits of America; The Patron Spirits Company; The Standard Meat Company; Thomas Financial Group; Trinchero Family Estates; USF Health; Robert & Nelda VanSchoik

**Silver Sponsors-** American Beverage Marketers; Anna Maria Oyster Barr; Bacardi; Berman and Company; Brown Forman Beverages & Kerry Group; Constellation Wines; Foley Family Wines; Francis Ford Coppola Winery; Laser Spine Institute; Majestic Fine Wines & Premier Beverage; Moet Hennessy USA; Monin Gourmet Flavorings; Red Bull; Remy Cointreau USA; Sazerac/ Gemini Spirits and Wine; Skyy Spirits; Ste Michelle Wine Estates; Verizon Wireless; W.J. Deutsch & Sons, Ltd; William Grant & Sons, Inc.

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Request a Fundraising Kit  
[www.info@cureFA.org](mailto:www.info@cureFA.org)

### Setting the Stage for Fundraising Growth

By Juliann Green



There is no question that 2009 has been a difficult year for non-profit fundraising, but FARA has met the challenge head on. With the support of our corporate partners and FARA families, friends and their supporters, FARA has managed to do what many other non-profits have not — increase our fundraising over last year. We thank all of you who have made this possible.

As we analyze FARA's fundraising challenges and opportunities, here are the areas that we identified and addressed in 2009 and plan to emphasize even more in 2010:

1) *FARA's success in moving basic research projects into the translational and clinical phases (see Ron Bartek's President's letter) along with our continued need to grow our research pipeline and strengthen our infrastructure requires a significant increase in funding over prior years. Work is well underway to heighten our fundraising programs.*

2) *Most of FARA's funds come from large numbers of small donations through events and personal fundraising, as opposed to most non-profits, which on average receive 90% of their funds from 10% of their donors. We are fortunate to have a dedicated and growing group of grassroots fundraising leaders from within the FARA family and patient community, along with a few significant corporate partners. While this grassroots funding will continue to be a critical component of our fundraising strategy, it is not sufficient to cover our increased funding needs.*

3) *To reach our goal of significantly increasing annual research funds, FARA needs to increase awareness of our brand and engage more large donors and corporate support.*

#### **So how do we get there?**

FARA took a significant step in 2009 with the formation of a Development Advisory Board. The nearly twenty business and industry leaders in this group have graciously agreed to be FARA's ambassadors. In the course of their work and personal endeavors, they will identify opportunities for FARA to tell its compelling story and make valuable connections to help us achieve our research and fundraising initiatives. Their names and organizations are listed on our website under Leadership. Along with our Development Committee, I will be working closely with them on nurturing each opportunity and relationship.

In addition to the introductions we hope our Development Advisory Board will bring us, we hope all of you will connect FARA with individual and corporate donors who may have an interest in hearing the FARA story. It is entirely possible that you have a business or personal connection that could be a significant FARA donor.

I also encourage you to consider giving monthly through

our monthly automatic deduction from your credit card. This method of giving offers benefits for both donor and non-profit.

Lastly, I know many of you have done a wonderful job of building a group of friends and colleagues who support your FARA fundraising efforts. You may want to personalize Ron's letter and send it to them, as well. While we are all concerned about asking too much, especially from our closest family and friends, remember that we need to keep them informed, too. If they don't know, they can't give. Individuals are much more likely to give and to give more significantly if they are asked by a person they know.

As I have traveled these past several months and met many of our fundraising families and supporters, I am humbled and inspired by your passion and commitment to raising funds for FA research. Please know that I am a phone call away to support you in any way that I can. ■

#### **Upcoming Events**

##### **January 18, 2010**

Play for FA- Family Fitness Night  
Rochester, NY

##### **February 20, 2010**

Outback Luncheon w/ The Zies Family  
Maumee, OH

##### **March 10 & 13, 2010**

Luebbe Dodgeball Tournament & Dinner Dance  
Liberty Township, OH

##### **April 12-18, 2010**

Outback Pro-Am  
Tampa, FL

##### **April 23, 2010**

Holly's Hope Golf Tournament  
Bedford, NH

##### **May 15-16, 2010**

Ham Supper & Walk-a-thon  
Jonestown, PA

##### **May 15-16, 2010**

Ride Ataxia NorCAL  
Sacramento, CA

\*Please check the FARA website [www.cureFA.org](http://www.cureFA.org) for a full list of events.

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

**Contributors:** Rob Bartek, Kyle Bryant, Felicia DeRosa, Marilyn Downing, Jennifer Farmer, Juliann Green, Paul Marcotte

**Editor:** Karen Smaalders

**Design/ Layout:** Anne Myers

Interview with Allison Rice, 30

### **When were you diagnosed with FA?**

When I was in high school I was very involved in sports, specifically softball and skiing. One year I got cut from the softball team and I noticed that my skills were going downhill. When I was 19 I developed a tremor in my right hand and I started eating with my left hand. I finally visited the doctor when I was a junior in college and he had never seen FA, so he dismissed it and told me it was “all in my head.” I was taking a college course in anatomy at the time and we read a bit about the central nervous system. All the symptoms matched the ones in the textbook so I knew I had ataxia but had to wait for a diagnosis to find out which type it was.

### **Where did you go to school?**

University of Vermont, here in Burlington.

### **Isn't it really cold there?**

Yes, we have snow from mid-December through March. However, I love the snow because I started doing a lot of adaptive snow skiing last year after about 10 years of being away from it. I love it – to be outside again is awesome! I went almost every weekend last winter – I racked up 10 ski days! My only regret is that I did not get into it years ago.

### **What were some of your concerns at school?**

My major was Nuclear Medicine Technology, which requires you to give patients radioactive injections. Because I was dealing with a fine motor skill issue, I was not able to give patients injections.

### **How did you confront that issue?**

Very soon after my diagnosis I had to be up front and let my professors know what was going on. It was hard, especially being newly diagnosed, to be up front about the issue.

### **How did it turn out?**

It was all just fine. We just had someone else perform the injections or a nurse would start an IV and I would administer the medicine through the IV. My professors made those small accommodations for me and we kept moving forward.

### **What were some of your concerns during your job search?**

I was unstable and wobbly during interviews and I was not comfortable talking about my condition. It turned into a situation in which everyone was uncomfortable and I never



got a call back. Then I got some advice from a previous professor who suggested that I change my approach and be up front about it. When I was eventually able to confront the issue and be honest about it, the tension was relieved, I have had several jobs since then.

### **What do you do now?**

I am a research coordinator at Fletcher Allen Health Care, which is the hospital affiliated with the University of Vermont. I run clinical trials, which involves a lot of patient care – giving medications, taking vital signs and doing EKGs – and asking a lot of questions. My job also includes a lot of administrative work with drug companies and various regulatory boards.

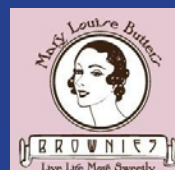
### **What do you love about your job?**

I love the patient care aspect of it. I work in clinical trials with Alzheimer's patients and I think they identify with the physical aspect of my condition. I enjoy connecting with my patients.

### **Why is your job important to you?**

I believe I am doing important work. The new Alzheimer's trial we are running is cutting-edge science. The trial is for an IV-infusion drug which is administered once a month because Alzheimer's patients typically have a hard time remembering to take their meds daily. There are a few different theories about Alzheimer's, and this trial will either prove or disprove one of the theories. Either way, it's a turning point in Alzheimer's research. This trial will help solve the mystery of Alzheimer's disease. ■

**If you have FA, please consider sharing your story. We want to hear about your greatest challenges and achievements. Whatever your age, you have an inspirational story to tell! If you have an idea or a submission for Living with FA, please contact [info@CureFA.org](mailto:info@CureFA.org).**



Inspired by the courage of two young women, Sara and Laura Ferrarone from Rochester, NY and the many others with Friedreich's Ataxia (FA), Mary Louise Butters created a signature FARA Brownie especially for the Friedreich's Ataxia Research Alliance. Consider supporting the cause with your holiday and corporate gift giving. Visit [ButtersBrownies.com](http://ButtersBrownies.com) or call 512-922-0342. Use the promotional code “FARA” to receive 10% off your order.

Special thanks to all of our grassroots event planners and their volunteer committees. These invaluable volunteers work tirelessly to host fun and entertaining events that raise both critical awareness and funds for FARA. Raising \$1.6 million as a collective group, these event planners are the driving force behind Friedreich's ataxia research advancement.

2009 EVENTS	EVENT PLANNERS	LOCATION	IN HONOR OF	AMOUNT RAISED
<b>NORTHEAST</b>				
*Benefit Dance/ Irish Music	Barbara McCaffrey	Norwood, MA	Barbara McCaffrey	\$9,100
The Bullpen Open Golf Tournament	Jason Krogmann	Saratoga Springs, NY	Dylan McDonnell	\$3,145
Find A Cure Dinner/ Auction	Mary Caruso	Branford, CT	Sam and Alex Bode	\$27,000
*Fox Chapel Area High School Orchestral Concert	Mairi Thompson	Pittsburgh, PA	Lauren Krivinko	\$4,500
*Friends Fighting FA- Tapas Event , Alec's Walk, & Play for FA: Family Fitness Night	Karen Brown & Kim Bellnier	Rochester, NY	Alec & Colin Brown	\$3,500
Fuzzy Buzzy Golf Tournament	Paul & Maureen Stanieich	Windham, NH	Erin O'Neil	\$10,000
*Holly's Hope Talent Show, Bracelets, & Thrill the World	Hannelore Federspill & Lisa Wojenski	Bedford, NH	Holly Franz	\$11,250
*Ironman Send Off Outback Luncheon & Competition	Simon Hurley	Dewitt, NY	Linda Johnson	\$4,900
*Jacob's Garden Tractor Pull	Jacob Harmon	Orange, CT	Jacob Harmon	\$1,126
*Leonard Family Letter Writing Campaign	Keith Leonard	Chesapeake, VA	Luke Leonard	\$4,850
Music Festival	Dylan and Dave McDonnell	Glens Falls, NY	Dylan McDonnell	\$12,700
Olsen Event	Dan Olsen	Monroe, NJ	Dan Olsen	TBA
*Ride Ataxia Philadelphia	FARA & Outback Steakhouse	Limerick, PA	FA Community	\$120,000
Stars for a Night. . . A Glimmer of Hope Party	Dave Brown & Mary Hallett	Rochester, NY	Alec & Colin Brown	\$15,500
*St John's UCC of Jonestown Walk for a Cure & Ham Dinner	Dennis Wood	Jonestown, PA	Dennis Wood	\$6,625
Welsh Backyard Bash III	Peter and Francine Welsh	Harrisburg, PA	Brendan Welsh	\$18,030
Westchester Triathlon –Team Donovan	Norm & Debra Simpson Jennifer Sinnott	Rye, NY	Donovan Simpson	\$25,210
<b>SOUTHEAST</b>				
*Early Family Concert	Marie Early	Herndon, VA	Alexa Early	\$2,330
*FARA Ace for the Cure	Sandy Callaghan	Tampa, FL	FA Community	TBA
*FARA Energy Ball	The Avery Family	Tampa, FL	FA Community	\$900,000
*FARAthon Marine Corp Marathon & Kickball Tournament	Laura & Julie Cernosek	Washington DC	John Cernosek	\$15,700
Golf Tournament and Benefit Bash	Nelda Van Schoick	Bogart, GA	Robbi and Becca Van Schoick	\$60,000
<b>MIDWEST</b>				
5 <sup>th</sup> Annual Race for a Cure	Tammy Luebbe	Cincinnati, OH	Evan Luebbe	\$25,375
*Chicago Marathon	John Lagedrost & Matt Goldberg	Chicago, IL	Joanna Lagedrost	\$13,180
*Holly's Hope Party	Rob Franz	Spring Grove, IL	Holly Franz	\$4,500
Hole Out for a Cure- Rick Peters Charity Golf Tournament	Rick Peters	Peoria, IL	Rick and Todd Peters	\$20,860
*Hedrick Rotary Dinner	Gene Hedrick	Indiana	Grace Haupt	\$5,225
*I'm with AJ- Family Day	Donna Davis	Fayetteville, AR	AJ Davis	\$3,500
*Young Family's Barn Dance	Becky Young	Saybrook, IL	Emily Young	\$19,700
<b>NORTHWEST</b>				
*Oscar Lopez Pavlo Concert	Alefantis Family	Calgary, Canada	FA Community	TBA
Ride Ataxia III Cycling	Kyle Bryant	Portland, OR to Seattle, WA	FA Community	\$260,000
Ride Ataxia NorCal Party	Angela Lacativo Greene	Martinez, CA	Kyle Bryant	\$10,000
<b>SOUTHWEST</b>				
FA-ith (FA in the Heart) Dinner dance	Rupel Family	Sunnyvale, CA	Matt Rupel	\$60,000
Lone Star Benefit Bash/Castle Hills Golf Tournament	Vince Palasota Jerry Russell	Lewisville, TX	Robbi and Becca Van Schoick	TBA
San Francisco Bay Area Walk	Trent Gaylord	San Francisco, CA	Brianne Konanz	\$2,185
Third Annual Race 4 Results, Macy's Shop for a Cure, Stuff on a Stick Booth, Steps 4 a Cure	Carrie Laird	Bakersfield, CA	Jerod Laird	>\$12,000
Stephanie's Hope Holiday Boutique	Sharon Magness	Valencia, CA	Stephanie Magness	\$5,000

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

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



Jerod Laird's hard-working friends Zach & Nicole Schorr at the FARA "Stuff on a Stick" booth at the Harvest Festival in Bakersfield, CA



Carrie Laird greets the shoppers at Macy's "Shop for a Cure," which raised over \$1,000 for FARA in Bakersfield, CA



Brendan Welsh, helping out with his family's third annual Backyard Bash in Harrisburg, PA, is pleased to see that Outback is hard at work, cooking for the Welsh family's guests.



Buddies John Lagedrost and Matt Goldberg raised over \$10,000 by running in the Chicago Marathon in honor of John's sister, Joanna, who has FA. John, a junior at Cornell University along with Matt, is from Hinsdale, IL.



Event planner Trent Gaylord, alongside Brienne Konanz (center), is surrounded by the generous walkers from the San Francisco Bay Area FA Walk, held in August.



Ride Ataxia Founder Kyle Bryant's cousins, Angela and Ricky Greene, entertain guests such as these—Sue and Paul Konanz, Bart Rupel and Diane Bryant. The event was held at the Martinez Marina in Northern California.



Dancing to break the Guinness "Thrill the World" Record, these students raised money for FA research in honor of classmate Holly Franz.



Holly Franz and Jenn Sutton meet at the Holly's Hope event held in June at Captain's Quarters in Antioch, Illinois.



At the 7th annual Find A Cure event (Branford, CT), the creative team from The Cake Eaters received the 1st annual Friend and Ally award for their support in helping spread awareness of Friedreich's ataxia. Shown in photo is Mary Stuart Masterson, Jayce Bartok, Jesse Scoloro, Patrick and Carol Morris.



Mary Stuart Masterson, director of The Cake Eaters and dedicated supporter of FARA's goals, is shown here with Mary Caruso and her daughters Sam and Alex Bode, at their Find A Cure event September 11th.



Comfortably traveling the course at the Fuzzy Buzzy golf tournament are Steve Parsons, Christian Saurman, Jeff Baxter, Marcy Saurman, Amy Theriault, Erin O'Neil and Katelyn Campbell.



Area Charleston Outbacks, led by Tony Cajuno, organized a Bridge Walk, which was attended by the Van Schoick family from Georgia. Becca and Robbi Van Schoick are shown here with the lively Outback group.



Some of the crew members and the honorees posing for a photo prior to participating in the Westchester Triathlon, as members of Team Donovan. Donovan Simpson, of Yonkers, NY, is the VIP at this event.



The Young Family at their Barnraiser Fundraising event in Saybrook, Illinois.



Curtis Wong and Ron Duckstein with the Simpson Family at Heineken with a Heart in New York.

Outback Steakhouse and Heineken teamed up to host a series of happy hour events in major cities across the country to benefit FARA. The wonderfully successful campaign, **Heineken with a Heart**, featured donated Outback appetizers, Heineken product, music and a raffle at Outback locations in Glendale, CA; Buckhead, GA; Las Vegas, NV; Baltimore, MD; Chicago, IL; St. Petersburg, FL; Houston, TX. In addition to the funds and awareness raised by this great event series, it brought many FA families together for an evening of fellowship. Special thanks to Outback and Heineken for their warm hospitality and generous HEART! Watch the FARA website and FARA Facebook Fan page for the announcement of the 2010 event locations.



Heineken and Outback present FARA with a generous contribution of Heineken with a Heart proceeds in Atlanta, Georgia.



Laura Beth Jacquin and friends at the Heineken with a Heart event in Buckhead, Georgia.

## Contact Us



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