

From the Executive Director



Building Clinical Research Infrastructure

By Jennifer Farmer

What can a rare disease research-focused advocacy group do to move the needle on advancing treatments? What research gaps can a modest sized organization effectively fill? What can FARA do to make Friedreich's ataxia an attractive disease for industry partners to invest in and develop treatments for?

FARA is constantly asking itself these questions and assessing the internal and external needs of the FA research community. In March through June FARA President Ron Bartek and I had the opportunity to attend meetings with other rare and neurodegenerative disease groups where these questions were discussed in a broader context across diseases with external stakeholders such as the National Institutes of Health, the Food and Drug Administration, pharmaceutical and biotech representatives, and academic scientists and physicians.

There was clear consensus among all stakeholders and advisors on one area – clinical research infrastructure (i.e., patient registries and natural history studies). The good news is that FARA and the FA community recognized long ago the need for establishing a clinical research infrastructure. In fact, at these meetings FARA was asked to participate by sharing our experience with getting a patient registry and clinical research infrastructure established. This opportunity gave us a chance to reflect on how we have gotten to this point, and more importantly, to evaluate what aspects work well and what aspects need more of our attention.

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Largest FA Scientific Conference Held in Strasbourg, France May 5-7

By Jennifer Farmer

More than 200 scientists, physicians, and advocacy partners convened at The Institute of Genetics and Molecular and Cellular Biology (IGBMC) in Strasbourg, France to share their latest research on Friedreich's ataxia. The IGBMC was a perfect place to hold such a meeting because it is home to several researchers who have longstanding commitments and contributions to FA research. These researchers are part of the core group who identified the FRDA gene and mutation which cause FA, developed the first animal models, and now continue to do important research into understanding frataxin function, mitochondrial dysfunction in FA, and development of new animal and cellular models.

The Strasbourg meeting was the largest gathering of the FA scientific community and included:

- *Scientists from 15 countries*
- *Seven pharmaceutical companies participating through attendance, presentations of research, and/or sponsorship*
- *Six biotechnology companies contributing through sponsorship*
- *10 advocacy partners supporting the conference through sponsorship and/or attendance*
- *58 oral presentations and 53 poster presentations of original research*

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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.



Dear friends,

You will probably agree that the first half of 2011 has provided us all with encouraging signs of progress towards our goal. I would like to use one of those signs – the positive announcement of preliminary results from the phase II clinical trial of EPI-A0001 – to illustrate the growing strength of the FA community and the power of the partnerships we have built. I also want to thank you for the important roles you are playing in moving this and the other exciting therapeutic candidates forward toward treatments for FA.

FARA's involvement in the development of A0001 began in 2005. Edison's CEO, Dr. Guy Miller, briefed FARA on the company's efforts to advance molecules that would improve mitochondrial function in FA and related diseases. He asked FARA for advice on scientists that might be willing to test Edison's molecules in their FA cell assays. The FA scientist that agreed to conduct those tests informed FARA later that one of the Edison molecules had performed far better on his FA assay than any other molecule he had tested. Excited by that finding, FARA encouraged Edison to apply to the newly announced NIH Rapid Access to Intervention Development (RAID) pilot program. RAID required participation by a drug company, an academic investigator and a patient advocacy organization, so Edison, Dr. Robert Wilson of the University of Pennsylvania (the FA scientist who had tested the Edison molecules), and FARA co-applied. The result was that FA became the first rare disease to be accepted into the new program and RAID support helped accelerate the clinical development of this drug – EPI-A0001.

Knowing that further development of EPI-A0001 and subsequent Edison molecules aimed at mitochondrial function would be costly, Edison applied to FARA and a number of other research-funding organizations for additional support. After scientific peer reviews found great merit in the Edison proposal, FARA's Board of Directors decided, like the NIH, to support Edison's A0001 program. FARA awarded Edison two large research grants in 2005 and invested in Edison's preferred shares. Such support enabled Edison to advance A0001 to the stage of licensing clinical development of the molecule to Penwest Pharmaceuticals in 2007 and continuing its own research on additional mitochondrial drugs.

Penwest initiated the phase I clinical trial of A0001 in 2008 and, following active collaboration with FARA, launched the phase II trial of A0001 at the FARA clinical research network site at the Children's Hospital of Philadelphia in 2010. FARA used its patient registry to recruit the patients needed for the phase II trial and the FA community responded as always

– quickly and effectively. You saw in June the preliminary report from that trial – in both the low and high dose groups as compared to the placebo group, A0001 improved neurological function as measured by the Friedreich's Ataxia Rating Scale (FARS).

Preparations are now well under way for the crucial next steps in this drug's clinical development. David Lynch, the principal investigator of the trial, said, "We are working closely with Edison, the U.S. Food and Drug Administration (FDA) and our colleagues in FARA's Collaborative Clinical Research Network in Friedreich's Ataxia to design extended duration clinical trials to verify the encouraging data from A0001's phase II trial."

Before completing the phase II trial of A0001, Penwest was acquired by Endo Pharmaceuticals. In acquiring Penwest, Endo was focused on key elements for its portfolio of pain medications and, despite the positive outcome of the phase II trial of A0001, terminated the Penwest license agreement and returned the drug to Edison. In the meantime, Edison had continued apace to develop another mitochondrial molecule – EPI-743.

(To view FARA's press release, [click here.](#))

So, as a result of your longstanding, generous support and your active commitment to participating in clinical research, the FA community now has in clinical development two exciting drugs that show real promise for improving mitochondrial function in FA patients. At the same time, clinical and pre-clinical research continues to gain momentum in several other therapeutic approaches such as HDAC inhibition, EPO, and iron chelation, etc.

None of this progress would be possible without your support and participation and without the public-private partnerships that are proving to be so effective as FA patients, patient families, FA scientists, drug companies, government agencies, FARA, and FARA's worldwide advocacy colleagues work closely together. Those partnerships and the passion and commitment we all bring to them will treat and cure FA.

Thank you and warm regards,
Ron

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

So What is Clinical Research Infrastructure and Why is It So Important?

Clinical research infrastructure refers to the resources needed to facilitate any type of research, including clinical trials, that involve patients. These resources can include things like:

Patient registry: A database that captures information on individuals with a specific condition

FARA Patient Registry: www.curefa.org/registry

Natural history study: Longitudinal data (usually about 10 years) on individuals with a disease that describes and quantifies the progression of the disease along with the symptoms and manifestations of the disease. Natural history can sometimes serve as the basis from which measurements can be made to determine effects of new treatments, drugs or interventions.

Clinical outcome measures: Functional performance tests (e.g., timed pegboard or walk tests, vision, hearing or speech tests) that quantify how much change takes place in a specific amount of time and are used in clinical trials to measure whether a drug is altering the course of the disease.

Biomarkers: Anything that can be used as an indicator of a particular disease state — usually proteins, enzymes, genetic variants, imaging (MRI, CT or PET scans). Biomarkers can be used to assess risk of disease, diagnosis, or outcomes. Use of biomarkers in drug development is of great interest because biomarkers can provide evidence of biological activity, potentially demonstrating therapeutic benefit more quickly than traditional outcome measures.

Biorepository: A repository or bank of stored biological materials such as blood samples, DNA, organs, and tissues (such as skin, muscle, heart) that can be used for research.

Collaborative Clinical Research Network in FA (CCRN in FA): An international network of 11 clinical research centers that work together to advance treatments and clinical care for individuals with Friedreich's ataxia. Having such a network means that there are trained physicians and research coordinators ready to do clinical research studies and trials. Also, this network is backed by a data coordination center that facilitates all aspects of data collection, database management, and statistical analysis of study data. To learn more, visit www.curefa.org/network.html

We started off with smaller well-defined projects, like establishing a patient registry, ataxia scales, and clinical outcome measures. For example, initial discussions began

back in 2003 and 2004 when physician scientists started advising FARA of the need for clinical outcome measures. A group of FA physicians agreed on several different outcome measures and ataxia scales that were reasonable to test in FA. They designed a study that was funded by MDA and FARA and a faithful group of IT volunteers designed the database for the study. That initial study grew and evolved over the years to become much more than a single study of a few clinical outcome measures but a network of clinical research centers — the CCRN in FA. Through the CCRN in FA we have collected six years of natural history data (ongoing) in more than 600 individuals with FA, validated clinical outcome measures and the FARS scale, studied speech, vision and hearing, launched biomarker studies, established DNA and RNA repositories, and provided many blood samples to researchers around the world. The CCRN in FA investigators have been involved in multiple clinical trials including a few that were designed and conducted solely through network sites.

On a recent trip to Nashville I organized an effort to reach out to the FA community to collect blood samples for research studies and for our biorepository. Using the FARA patient registry I contacted individuals in the geographic area inviting them to participate in this research day. A research coordinator from Children's Hospital of Philadelphia accompanied me to help collect information, samples, and data. In a single six hours we collected blood samples, cheek swabs, medical history information and functional measures from more than 20 people for eight different research studies being conducted at multiple labs around the country. Yes, we were drawing about 10 tubes from each person and dividing them among various projects! Imagine if each of those eight research studies had to collect samples individually how much more time and effort would be involved.

New Clinical Resource — Special Initiative Funded by FARA Directors and Staff

There is an urgent need to identify and document clinical practice to guide clinicians in the management of people with FA. Application of clinical practice guidelines will provide improvement and consistency in health outcomes and thus quality of life for people with FA and also identify gaps in evidence that will present opportunities for further research. FARA's directors and staff felt that this project was a top priority because it could immediately lead to improvements, so they all agreed to fully support the project by pledging the required funding.

Dr. Martin Delatycki, Principal Investigator, and Louise Corben, Coordinator, at the CCRN site in Melbourne Australia

are leading a global effort to assemble the clinical management guidelines for FA. Clinical management guidelines define the diagnostic tests and criteria and the medical management or treatment modalities used in the diagnosis and treatment of a specific disease. These guidelines contain recommendations that are usually based on evidence from the studies in the medical literature, or if such evidence is lacking, guidelines can be written based on expert consensus/opinion. Physicians from all specialty areas (neurology, cardiology, orthopedics, genetics, etc.) and health professionals (physical, occupational and speech therapists) from around the world are collaborating to write these guidelines. The kick-off meeting for the guidelines project happened May 5 just prior to the start of the 4th International FA Scientific Conference. The guidelines will be completed in one year and available to individuals with FA, physicians and the general public. With a lot of effort, volunteer support, collaboration and partnership, and leadership from our entire FA community, we have established what many other rare disease groups are scrambling to achieve. We should see the benefits from these efforts in the near future in the form of improved understanding and management of FA symptoms, new biomarkers identified to assist in drug development, improved data and resources available for planning of clinical trials, and shorter timelines to launching studies. As new therapies become approved, this clinical research infrastructure will also be a vehicle to provide access and information about such treatment to individuals with FA. ■

FA Scientific Conference

(continued from page 1)

- 25 scholarships awarded to junior investigators submitting competitive abstracts to support their participation in the conference

In a packed conference room with people lining the steps, the conference began with a personal message delivered via video from FARA spokesman Kyle Bryant. Kyle encouraged conference participants to share their knowledge and ideas and build collaborations to bring us closer to treatments and a cure for FA. He also told the researchers that the FA community appreciates their dedication and commitment and backs them 100% by raising funds to support their research and participating in the clinical trials and studies.

[Click here to hear Kyle's talk on YouTube.](#)

Through three days of presentations and discussions the room remained packed and the audience stayed attentive and inquisitive. During the breaks and on tram rides in the early morning and evening it was obvious that this group

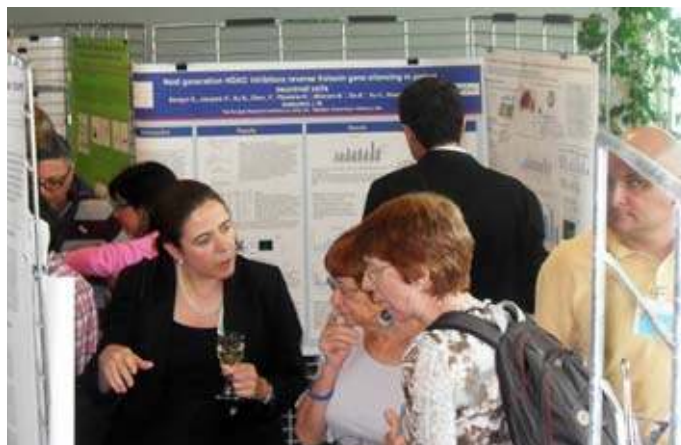
was benefiting from the opportunity to network, share ideas, and build collaborations. It was most exciting to see smaller groups huddled together engaging in animated and deeper discussions of their research.

Seeing so many new faces who have joined the field and the number and caliber of young investigators surprised many of the researchers who had attended the three previous international conferences. Graduate students, postdocs, and young investigators delivered many of the oral and poster presentations. These newcomers bring an energizing passion and intensity for science, and their involvement makes the future of FA research bright.

This conference covered all aspects of FA research from the intricacies of how the GAA repeat expansion in the FRDA gene results in lower frataxin protein to results of clinical trials. The conference was divided into eight sessions based on topic area, but all attendees participated equally throughout as insights from one area of the research may have implications and insights into another area. Basic researchers and clinical scientists shared their findings, ideas and challenges so that each could learn from the applied knowledge.

Genetics and Frataxin

The genetics and frataxin sessions featured highly detailed and technical presentations. Genetics took a deep dive into the complex process of going from DNA sequence to a single gene (FRDA) with a GAA repeat mutation to protein (frataxin) and all of the structural, functional, and regulatory genetic components involved. For example, several presentations focused on studies of how the GAA repeats and specific control elements that can either stabilize repeats or make them expand. These presentations also looked at the mitochondria and studies investigating how Iron sulphur clusters are assembled and the precise role of frataxin in that process. It is easy to feel overwhelmed by the detail of the experiments and data presented; however, there is great satisfaction in



Scientists discuss work presented during the poster sessions at the 4th International Scientific Conference in France.



Ron, Dr. Giovanni Manfredi and Dr. Helene Puccio strategize on how to fit more into a busy conference schedule.

understanding the mechanisms and processes, of how things work, and when our research community reaches consensus. Discussions included the application of these research findings and insights to open the door to new avenues of treatment targets.

Drug Discovery Assays

More than six groups reported on assays (tests) developed specifically for drug discovery. Each assay is unique in what it measures or targets. Many of these groups presented on the design of their assay and initial application of the assays in screening libraries of compounds. In addition, several groups presented on very targeted drug discovery efforts in which a specific type of compound or therapy is being developed and tested in the laboratory with the goal of creating a novel therapeutic candidate. With libraries of compounds being screened and drug discovery teams designing novel compounds, there is confidence that new therapies will continue to emerge in the very near term.

Cellular and Animal Models

Developing new cellular and animal models of FA is a competitive, diverse, and ripe field of research. There were more abstract submissions for this session than any other – and this is a good thing. Yeast, fly, worm, and mouse models continue to be developed for researchers to test hypotheses about how things work (or don't work) and for identifying and testing compounds or therapeutic candidates. New human FA cell models of neurons or cardiac cells are now emerging. While setting up for the conference at IGBMC, Dr. Puccio gave us an inside peek through the microscope at her patient-derived iPS cells and cardiac cells. When we peered down the microscope we could see this beautiful group of cells organized as a unit, beating! Truly amazing. All of these models are highly valuable as they are the tools needed to test new compounds and understand how they work. Well-established and -charac-

terized cell and animal models will accelerate the research process to clinical trials.

Clinical Research

Clinical discoveries and research continue to provide critical insights. A researcher presented the first report of an individual with two point mutations in the FRDA gene, reminding us that diagnosis of FA is not always straightforward. Such rare mutations also provide new insights into the function of frataxin. Reports were shared on clinical research in which individuals with FA volunteered for studies on diabetes, vision, hearing, speech, brain function, and frataxin measurement and genetic studies. These studies make important contributions to our understanding how dysfunction at the level of gene and protein cause symptoms and disease in individuals, how to better treat such symptoms, and ways to identify biomarkers or measures of FA that can be used in future clinical trials to demonstrate efficacy of treatments. Results from clinical trials of Idebenone, Deferiprone and Erythropoietin were presented and discussed. While we hope for conclusive results in the form of evidence of treatment or clinical benefit from these trials, not all trials are designed for this purpose. Early stage clinical trials are designed to inform us about safety, dosage, and/or biological activity. Even when results fall short of expectations there is still a great deal of knowledge gained and lessons learned from the trials that inform us going forward.

There were so many highlights from the presentations and discussions that it is difficult to select only a few to share. All of the scientific abstracts and slideshow of photos from the conference can be accessed on the FARA website at www.curefa.org/conference.html

The proceedings of the conference will be published in *Disease Models and Mechanisms* by Drs. Alain Martelli and Marek Napierala. ■



It was a packed house at the IGBMC in Strasbourg, France for the 4th International Scientific Conference.

A Major FARA Funding Initiative: Stem Cells as Disease Models and Promising Therapies

By Bronya Keats, PhD

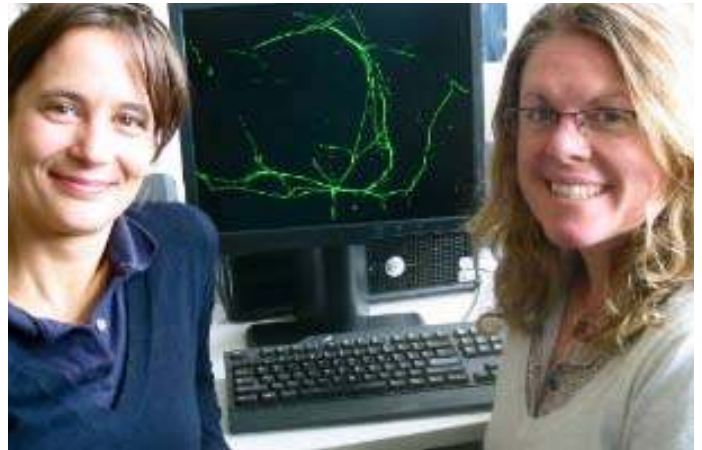
FARA's commitment to funding and nurturing research and resources critical for advancing promising therapies for FA is exemplified by its support of projects worldwide that are focused on the development and distribution of standardized mouse and cellular models of FA. These models are valuable resources both for discovering and for testing new drugs. Through a contractual agreement between FARA and the Jackson Laboratory, and with guidance from the FA Mouse Models Task Force, FA mouse models are being evaluated, improved, characterized and distributed to the FA research community. The availability of these standardized models not only reduces the amount of work that needs to be done in the researchers' laboratories, it ensures that meaningful comparisons of results from different laboratories are possible.

Precise, well-characterized cellular models are also a critical resource for advancing scientific knowledge. Cellular models for FA are taking a giant leap forward in large part because of FARA-funded research to generate FA-induced pluripotent stem (iPS) cells. These cells have many of the properties of embryonic stem cells, but a huge advantage is that iPS cells can be obtained from sources such as skin cells. Basically, the skin cells are reprogrammed such that they are capable of becoming virtually any cell type in the body. While numerous FA cell models have been used in research laboratories, none have been truly representative of FA. The discovery that skin cells, which are easy to obtain, can be reprogrammed into iPS cells is a tremendous breakthrough; it means that neuronal and cardiac cell lines from FA patients are now a reality.

FARA grants for stem cell work have been awarded to Drs. Mirella Dottori and Alice Pébay in Australia, Dr. Joel Gottesfeld in California, and Dr. Hélène Puccio in France. These investigators presented their findings to attendees at the International Friedreich's Ataxia Scientific Conference in May and also met as members of a Stem Cell Task Force, whose mission is to guide the standardized characterization of FA iPS cells and their distribution to the FA research community. In an interview conducted at the Scientific Conference, Dr. Mirella Dottori describes iPS cell work and its importance to FA research advancement.

[Click here to view the interview on YouTube.](#)

Two exciting papers on this subject were recently published: In the journal *Cell Stem Cell*, Dr. Gottesfeld and colleagues reported the results of their experiments dem-



Dr. Alice Pébay and Dr. Mirella Dottori flank an image of neurons of the peripheral nervous system that they generated from FA iPS cells.

onstrating that the FA iPS cells exhibit characteristics that are consistent with the disease, including instability of the GAA repeat expansion, and Drs. Dottori and Pébay and colleagues described their success with differentiating FA iPS cells into nerves and beating heart cells in the journal *Stem Cell Reviews and Reports*.

We need reproducible, stable, well-characterized FA iPS cell lines that are appropriate for research (including drug screening), maintained in a reputable facility, and distributed among researchers. Discussions are underway to establish this facility, which will derive, store and distribute high-quality iPS lines for pre-clinical and clinical research. This work will be done in accordance with strict standardized procedures and will accelerate progress in the development of cell therapies for FA. These well-characterized cellular models will be extremely useful for high throughput screening to discover potential therapeutic agents, and for more detailed testing of promising candidates. They will also provide precise models of the cell types that are primarily affected in FA (cardiomyocytes and dorsal root ganglion neurons), and thus, they are particularly valuable for studying the molecular and cellular pathology, as well as the progressive degeneration, in FA.

The potential use of iPS cells in the development of stem cell therapies is especially exciting. It would eliminate the problem of immune rejection that results from transplanting cells from a donor. Effective methods for correcting frataxin levels in these cells are being developed. In addition, the necessary technologies and methodologies for growing these cells to form replacement heart tissue are now in place and being pursued for FA. Certainly, there are hurdles that need to be overcome and further detailed scientific investigations of the properties of iPS cells are essential, but there is every reason to be cautiously optimistic. ■

UPS Campaign Exceeds \$30,000 for FARA

By Felicia DeRosa

As the Southern California district of the United Parcel Service prepared to ramp up for the 2010 United Way Campaign, UPS manager Kris Wilson approached Brian Lamasus about telling his family's story to the UPS management team. Kris knew that Brian's son Josh was diagnosed with Friedreich's ataxia in December 2009 at the age of 16. She also knew about FARA's work facilitating and advancing FA research. Brian welcomed the opportunity to tell his story. Following his presentation to the management team, division manager Mark Bergman asked Brian to share it with the rest of the division. Brian told his story to no less than 10 work groups and informed them about how making a donation to United Way and selecting FARA as the charity of choice could advance research to help his son. The United Way Campaign at the Ontario, California division of UPS became a new platform for Brian to educate co-workers about FARA and enlist their support. Brian's division took hold of this initiative, posting the FARA faces banner at the office during the campaign, and allowing Brian to distribute information about FARA during his talks to work groups



Brian Lamasus and his UPS coworker in front of one of the delivery trucks.

"It was a win, win situation," Brian said. "I was able to raise funds and awareness about this little-known disease and my coworkers felt good knowing that their contribution went to an effort that positively impacts someone close to them."


The response he received from the campaign was overwhelming. A total of 210 UPS employees collectively pledged over \$30,000 to FARA through the United Way! This was by far the largest donation that FARA has ever received through the United Way! "This outpouring of support was amazing," Brian said. "I am grateful that UPS fosters a culture of community support and that my team at UPS has come together

in such a meaningful way to advance research to benefit my son Josh and all people with FA."

If your workplace has an annual United Way campaign, please contact the administering chapter to add FARA to the list of recipient charities. You may also contact FARA at info@curefa.org for supporting informational materials. ■

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Database for Future
Clinical Trials:**

www.cureFA.org/registry

 The Children's Hospital of Philadelphia®
Hope lives here.

4TH ANNUAL FRIEDREICH'S ATAXIA SYMPOSIUM

Crowne Plaza
Hotel Valley Forge
260 Mall Blvd,
King of Prussia, PA 19406
**Saturday, October 8, 2011,
All Day**

Bringing together patients, families and healthcare professionals for a day of learning and camaraderie. Ride Ataxia Philly and the symposium will take place within close proximity of each other for easy access to both events. For more information, visit www.chop.edu/cme

Ride Ataxia Tops \$1 Million for Research

By Kyle Bryant

My family is not unlike other families who are affected by FA. When I was diagnosed we were devastated. We felt trapped by the haunting fear of what the future might hold for us with this disease. Over the course of several years, I moved through all the traditional stages of coping. I cried. I got angry. I ignored it. I was lost in "what could be" and stripped of my ability to take action.



In honor of his courage, confidence and leadership in the fight against FA, we dedicate Ride Ataxia NorCal 2011 to the life of Phillip Bennett.

My discovery of and transition to using a trike — this adaptation from two wheels to three — mirrored the shift in my own thinking. I decided there was no use sitting around imagining the worst case scenario. With the support of my family, I took action and wrote my own future. Even though this disease threatened to take all of my energy and severely limit my mobility, we decided it was time to act, so we rode our bikes across the country. You have likely heard this story before but I revisit it to draw the contrast between the total devastation we felt when I was diagnosed to the feeling we have now realizing the Kyle Bryant Translational Research award will top the \$1 million mark this year. Wow! It blows me away to think that we were nervous about setting our goal at \$40,000 for our first ride in 2007. We had no idea where the money was going to come from. We just knew one thing — we couldn't just sit around feeling sad, angry and lost anymore. My Mom says that fundraising is "our therapy" because we know we are contributing to the cause for ourselves and for many others.

Our decision for action brought us together with many like-minded FA families and their friends who also felt the push inside of them to act, ride, and contribute to funding research in pursuit of treatments and a cure. My family and friends joined together with these FA families and friends to reach this funding milestone.



Kyle Bryant & Team Bridgman enjoy another great ride together at Ride Ataxia NorCal.

As a community, we continue to write our own future through Ride Ataxia. This was our first year organizing a ride in Dallas. Thanks to the generous help of our partners at the Midwest Region of Outback Steakhouse, Blaise Hadley, Laura Pitzer and Mike Yates, we had 555 participants who raised \$180,000 for FARA — a new Ride Ataxia single-day event record! This dedicated Outback team who generously helped plan, fundraise, volunteer, and prepare delicious food for the pre-ride team dinner, rest stops and the post-ride lunch, made me a believer in the saying, "Everything is bigger in Texas." In NorCal, we were reunited with many of our long-standing teammates who all brought their friends, effectively growing the team and the circle of support to 300 participants, and raising more than \$100,000 for the second consecutive year. Ride Ataxia is off to a blazing start this year. I invite you to act, ride, fundraise and help end FA in Philadelphia on October 9 and Orlando on November 6. Together we will find a cure! ■



Outback Steakhouse presents a check for \$124,500 at Ride Ataxia Dallas. Thank you Blaise, Laura, Mike, and all the Outbackers in Texas and the central region!



Riders lineup for a great day Ride Ataxia NorCal.



Helpful Volunteers from University of California, Davis direct riders along their route at Ride Ataxia NorCal.



Cyclists line up and anxiously await the start of the 50-mile route at Ride Ataxia Dallas.



Attention all Ataxian Athletes!

Applications for the 2011 Ataxian Athlete Initiative (AAI) are now available! Whether you are an avid adaptive cyclist or a beginner with no experience, this is your opportunity to receive funding for your adaptive cycling equipment! Download your application at rideataxia.org/aai.php.

Application deadline: September 1, 2011.



Riders enjoy the beautiful weather and a great Ride in Dallas.



Team Izzy Boosts Team FARA

By Jamie Young

Team FARA is off to a great start this year with five events completed and four more scheduled for the remainder of 2011. Team FARA spreads awareness and raises funds for FARA through participation in any athletic endurance event, allowing FARA to reach new audiences and donors. Growing this program doesn't just raise more funds, but recruits higher participation at each event to create more awareness of the cause.

Team Izzy, who recently participated in the See Jane Run 5k in Alameda, California, illustrates the success of Team FARA. Team Izzy was started by Zoe and George Penston who were motivated by their 10-year-old daughter Izzy's spirit, strength and courage after her diagnosis of FA. Not long after Izzy was diagnosed, she set up shop in her

front yard selling lemonade to raise money for research. Inspired by Izzy's efforts, Zoe and George asked their friends and family to join Izzy's Team FARA.

Through sharing Izzy's story and the hope for the cure, the Penston family recruited over 50 Team Izzy members to join them at the 5K run dressed in FARA gear. The largest Team FARA event to date also raised over \$11,000 for research!

"Joining Team FARA and fundraising really gave me an outlet; it relieved some of the feelings of hopelessness that came with the diagnosis. It was also a way for us to show Izzy that we will always be fighting for her and doing all we can to raise awareness and be part of the solution. So that someday when she asks if the cure has been found, we'll be able to say yes. I honestly had no idea that we'd raise so much money! It's been amazing. We can't wait for our next fundraiser."

— Zoe Penston

You can join Team FARA simply by registering in a local athletic event of your choice and deciding to do so in FARA's name. FARA will provide a fundraising webpage for you to customize with a photo and information about your event participation. If Team FARA is competing in an event in your area, we encourage you to connect with the athletes to find out how you might support their efforts. For more information, please visit www.curefa.org/teamfara.html and contact info@curefa.org. ■

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

www.cureFA.org/registry

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Senior Fellow, Center for Medicine in the
Public Interest
Former Associate Commissioner, US Food
and Drug Administration

Joseph Pont, Director
Vice President Marketing and Key Account
Management, Lonza

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

UPCOMING EVENTS for 2011**JULY**

July 15-16, 2011
Get Fit for Charity
Dunedin, FL

July 15-17, 2011
FA Woodstock
La Porte, IN

July 28, 2011
Outback Luncheon
White Plains, NY

AUGUST

August 12, 2011
Welsh Family's 5th Annual
Bash in the Backyard for FARA
Harrisburg, PA

August 20, 2011
Young Family Bamraiser
Gibson City, IL

August 22, 2011
Peters Hole Out for a Cure
Peoria, IL

August 25-27, 2011
FARA Energy Ball
Tampa, FL

SEPTEMBER

September 11, 2011
Community Day to Cure FA
North Branford, CT

September 11, 2011
Team FARA — Chicago ½ Marathon
Chicago, IL

September 11, 2011
Team FARA
Team Annie Bananie
River Run Half Marathon and 5K

September 18, 2011
Fuzzy Buzzy Golf Tournament
Windham, NH

September 22, 2011
Century 21 Golf Tournament
Rancho Cucamonga, CA

September 24, 2011
Race for Grace
Bristol, RI

September 25, 2011
Team Donovan —
Jarden Westchester Triathlon
Rye, NY

OCTOBER

October 8, 2011
FA-ITH
St. Justin's Church
Santa Clara, CA

October 9, 2011
Ride Ataxia Philadelphia
Limerick, PA

October 9, 2011
Team Stephanie's Hope
Half Marathon
San Luis Obispo, CA

October 22, 2011
Swing Away at FA-
Wiffle Ball Tournament
Cumming, GA

NOVEMBER

November 6, 2011
Ride Ataxia Orlando
Claremont, FL

November 6, 2011
Team FARA — ING NYC Marathon
New York, NY

November 12, 2011
Stephanie's Hope
Santa Clarita, CA

November 20, 2011
Team FARA —
Philadelphia Marathon
Philadelphia, PA

**Please check the FARA
website for contact information
and a full list of events.**



THE FARA ENERGY BALL

August 25th
Cultivating a Cure
Scientific Symposium

August 26th
"Martinis & Poker"
Patron Party

August 27th
The FARA Energy Ball
with live performance
by Gin Blossoms

AUGUST 25-27, 2011
in Tampa, Florida.

To benefit FARA and
USF Ataxia Research Center

Book your tickets online now.

www.curefa.org/EnergyBall

Donations in lieu of attendance are
gratefully accepted.

Thanks to Our Generous Sponsors*

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**As of June 27, 2011*

Presenting Sponsors:



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Paul and Mary Jacobs



Donavan Simpson and Tiffany Gambill get ready for an evening of fun at Tiffany's Friends for FARA in Bridgewater, MA.



Roman Di Croce along with friends and family enjoys a delicious lunch at their Outback Luncheon Fundraiser in Tacoma, WA.



Team Shine completes the 7 Mile Bridge Run in the Florida Keys in Memory of Carol DeFilippo.



Charlie (Son of Carol) and Dana DeFilippo celebrate their Team FARA victory.



Anna Timbie prepares to participate in the Boston Marathon on behalf of Team FARA.



Some familiar faces party down on the dance floor at the Valentine's Day Ball.



Jade Perry addresses the crowd at the Valentine's Day Ball in Middleboro, MA.



Keith O'Brien and Friends lead the crowd at the New Jersey Seaside Stride.



The FARA community takes over Seaside, NJ for a great turnout at the Stride.



It's all smiles at the Play for FA Family Fitness Night in Hilton, NY.



Gabrielle Angiolelli donates to FARA from her Fundraiser Hockey Game at her college in Quebec, Canada.



Gabrielle Angiolelli receiving the check from the FARA Hockey Game Fundraiser she organized at her school in Quebec, Canada



GCMS school faculty came together to work the Charity Night Dinner at DJ Sullivan's in Gibson City, IL. in honor of Emily Young.



These hard working Ride Ataxia Dallas volunteers registered 555 cyclists the morning of the ride.



Erin O'Neil & the Couriers finish up a great meal at Erin's Flatbread Charity Night Fundraiser in Bedford, MA.



Vince Palesota, Kyle Bryant and Jerry Russell speaking to guests at the Lonestar Benefit Bash in Flower Mound, TX

The FARA Advocate is brought to you by:

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