SUMMER 2013

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

This Issue:

President's Message / Grants Update / Ride Ataxia
Treatment Pipeline / Living with FA

FARA Friedreich's Ataxia Research Alliance

www.cureFA.org

FARA Meeting Showcases Public-Private Partnerships

By Ron Bartek

Dear friends,

In several of these messages, I have emphasized the importance of public-private partnerships to accelerating our progress in treating and curing FA. Between June 6-8, 2013, in Silver Spring, Maryland, FARA held fantastic meetings that proved to be an impressive showcase of public-private partnership that demonstrated clearly how essential these partnerships are in accomplishing our mission.

The meetings were designed around FARA's Scientific Advisory Board, our Collaborative Clinical Research Network (CCRN) in FA, and the FARA Board of Directors. These three groups were joined by key representatives of our partners from government agencies, the biopharmaceutical industry, and other advocacy organizations. The 65 participants included 13 scientific advisors and FA investigators from the United States, Australia and Europe, 15 clinical investigators from CCRN sites in the United States and Australia, nine officials from the Food and Drug Administration (FDA), three from the National Institutes of Health (NIH), 16 representatives from a dozen biopharmaceutical companies and four colleagues from other advocacy organizations.

None were casual, passive, part-time participants. All of them actively participated during the entire set of meetings. Most of them made presentations and participated in the discussion periods. Several NIH and FDA officials even chaired sessions on such critical matters as clinical trial design and developing clinical outcome measures and biomarkers that might provide the basis for FDA approvals of FA therapies. They also briefed the group on NIH and FDA programs from which they encouraged our FA investigators and companies to seek support.

The participants from the biopharmaceutical industry ranged widely in terms of company size and type of current interest in FA. Some of these companies are already deeply involved in FA research and have drugs in our clinical trials. Others have drug candidates they are taking through pre-clinical development in preparation for clinical trials. Still others have expressed interest in FA drug candidates in early stage development — *e.g.*, the HDAC inhibitor and gene therapy. All of these industry representatives were deeply involved in the presentations, discussions and dinner conversations with FA scientists, CCRN investigators, government officials and FARA's staff, advisors and directors. All of them are staying in frequent contact with FARA and are clearly interested in full collaboration with us as they approach or conduct FA clinical trials.

One of the largest companies in the U.S. biopharmaceutical industry — Pfizer Pharmaceuticals — who participated fully in the meetings, has expressed interest in FA, and recently asked that FARA serve on the company's new Rare Disease Advocacy Advisory Board. Like all the companies with whom FARA is collaborating, Pfizer has made it clear that they are attracted to the FA community because of the public-private partnership business model FARA has been successful in building and the results that model is obtaining.

You are familiar with the FARA business model because you continue to help build it. It is founded on public-private partnership that pulls FA patients and families together with FA scientists and our colleagues from government agencies, industry, and other advocacy organizations. The results to date show that, together, we have been able to support the basic science that continues to push discoveries into the FA treatment pipeline; to develop the translational tools scientists and drug developers need to select the best drug candidates from the pipeline, as well as the clinical infrastructure drug companies need to pull those candidates out of the pipeline and into effective clinical trials that will lead to applications to our colleagues at the FDA for approvals.

As always, I would like to thank you for the vital role you play in all this progress. Because of the FARA business model you have helped build on the solid foundation of public-private partnerships (that was so much on proud display in the June meetings in Silver Spring), we can be absolutely confident that, together, we will treat and cure FA, and that we are clearly on track and accelerating toward accomplishing that mission.

Warm regards to you and your families,
Ron

Treatment Pipeline

By Jennifer Farmer

In the first 15 minutes of waking semi-consciousness between 5:45 and 6:00am, I sometimes see challenges and problems in a different way. Perhaps this semi-conscious state has not yet engaged "negative" thinking – when our brain tells us all the reasons we can't do something or all reasons something won't work. It is usually during this time that creative solutions to roadblocks in our research program percolate to the top.

I wake up excited each day and committed to advancing our research progress. This summer we will have three clinical trials enrolling subjects in the United States and several others in Europe. We also recently received encouraging results from completed studies. For example, the first HDAC inhibitor study demonstrated that even single doses of the drug could increase frataxin in subjects (measured in blood samples).

The resources needed to take a drug from the lab bench to doctor's office/pharmacy, where it is prescribed as a treatment, are large and diverse.

• ***At the early stages in the laboratory, it is important to have robust and validated cell and animal models for screening and testing new compounds – to figure out if you have a lead candidate.***

• ***Lead candidates then move to pre-clinical development; here there is a massive effort to better understand the candidate, evaluating chemistry, pharmacology and toxicology testing in multiple animal species, manufacturing of a drug and validating biomarkers.***

• ***During the pre-clinical stage, there is constant attention on moving to clinic. Significant efforts are put forward to plan not only the first clinical trial but actually develop a plan and design for trials that take the drug to late stage trials and approval.***

There is a path and process defined to some extent by key regulatory milestones. However, we know that for rare disease, like FA, the standard paradigm presents special challenges, some of which are directly related to limited human and financial resources and time. Because of these challenges, we must optimize our efforts, take what we learn from one study and apply it for the next, and have the scientific tools and clinical infrastructure in place that can be leveraged across all lead candidates.

Here is an update on some of the lead candidates in the treatment pipeline.

EPI-743 – In January 2013, Edison Pharmaceuticals announced the launch of a Phase 2 clinical trial of EPI-743 in adults with FA. There are three sites – University of South Florida, Children's Hospital of Philadelphia and University of California Los Angeles. As of June 2013, the trial was 2/3 enrolled and all subjects had been identified. Subjects were recruited through the FARA Patient Registry. The goal is to have enrollment completed (60 subjects) by the end of the summer. This is a double-blind, placebo controlled study. For more information on the study go to ***www.clinicaltrials.gov/ct2/show/NCT01728064***

OX1 - Viropharma is in the final stages of planning and launching a Phase 1 trial of OX1. This trial will be conducted in adults with FA at multiple sites around the US. The company hopes to announce the locations and details later this summer via the Patient Registry. As this is a Phase 1 study, the focus is on dosage and safety.

dPUFAs - FARA awarded a grant to Retrotope earlier this year to assist with the manufacturing of drug product and toxicology studies required for IND (Investigational New Drug) filing with the FDA. dPUFAs are deuterated polyunsaturated fatty acids. These compounds are known to protect cells from oxidative damage.

EGb761 – At the end of 2012, we removed EGb761 from the active pipeline due to the long duration of the study and lack of reported results. In April 2013, Ipsen posted results of a Phase 2 study, Efficacy of EGb761 in Patients Suffering from Friedreich's Ataxia. This was a placebo, controlled study of 22 subjects for 12-14 weeks. The full results are posted on ***www.clinicaltrials.gov/ct2/show/NCT00824512***

Unfortunately, due to the small number of subjects, the investigators were not able to do complete statistical analysis of all the outcome measures. The primary outcome measure was a biochemical measure based on exercise - creatine rephosphorylation rate post exercise measured using Phosphorus 31 Nuclear Magnetic Resonance (P-31 NMR) spectroscopy. There were no differences between the treatment and placebo group on this measure.

EPO – Studies of EPO and drug discovery of EPO-mimetics continue to be of interest as these compounds raise frataxin levels in

various models. Because the mechanism is not yet understood, FARA has funded a study to gain new insight into the workings of this compound. Due to small sample sizes or a lack of control arm, numerous clinical trials of EPO have shown mixed results and have been difficult to interpret. Dr. Francesco Sacca at the University Federico II in Naples, Italy, has designed and initiated a Phase 2 clinical trial of EPO. This is a double-blind, placebo controlled study titled Efficacy Study of Epoetin Alfa in Friedreich Ataxia (FRIEMAX) that is enrolling subjects at three sites in Italy. The study will test the effect of erythropoietin on exercise capacity, which is reduced in patients with FA. Additional objectives of the study will be the drug's safety and tolerability, and its effect on frataxin, blood vessel reactivity, heart functional indexes and disease progression. This study began in early 2013. In June, Dr. Sacca reported that 37 subjects had been enrolled, 17 more have been identified to enroll in the next few weeks and only 2 more patients are needed to fill the study. Subjects have come from various regions of Italy as well as Spain, Cyprus and Bulgaria. This study is being funded by FARA through the Keith Michael Andrus Memorial Award and AISA (Associazione Italiana per la lotta alle Sindromi Atassiche). ***ClinicalTrials.gov*** reference: ***http://www.clinicaltrials.gov/ct2/show/NCT01493973***

Resveratrol - Resveratrol has been under investigation as a compound that could improve mitochondrial function and, as some studies suggest, increase longevity, lowering glucose levels and anti-cancer activity. Researchers in Australia found that Resveratrol also increased frataxin levels in laboratory studies of cell and animal models. FARA funded an open-label, pilot study of Resveratrol at the University of Melbourne which began in April 2011. The study team reported the results at the American Academy of Neurology in April and FARA's Clinical Research Conference in June 2013. The primary objective of the study was to evaluate the safety, tolerability and efficacy of two different doses of Resveratrol (1g and 5g). 27 subjects were enrolled, and 24 completed the treatment for 12 weeks. Subjects were compared to baseline on primary and secondary outcome measures after the 12 weeks. The primary outcome measure of lymphocyte frataxin levels did not show a difference in either treatment group. Some of the secondary outcome measures, such as neurological rating scales, and speech measures, showed improvement in the high dose group; however, this should be interpreted with caution as there was no placebo group. The high dose group that received 5 grams of resveratrol per day had significant gastrointestinal adverse events including diarrhea, loose stools and abdominal pain. These interesting clinical findings suggest the need for further studies, such as a placebo controlled trial with additional dose groups. FARA has invited the study team to deliver a webinar with full details of the study and results later this summer.

Interferon gamma - Dr. Roberto Testi at the University of Rome "Tor Vergata" published a study in March 2012 that showed that Interferon gamma (IFNγ) increases frataxin levels in both cell and animal models of Friedreich's ataxia. The data in the report suggest that frataxin is increased by increased transcription of the FXN gene. Interferon gamma (Actimmune) is a drug approved for treatment of other rare diseases - chronic granulomatous disease (CGD) and severe, malignant osteopetrosis. Dr. Testi is planning to further evaluate IFNγ by conducting a pilot study in adults with FA. He will be evaluating three different doses of IFNγ and seeing if there is an effect on frataxin levels.

In addition, Dr. David Lynch, Principal Investigator of the Collaborative Clinical Research Network (CCRN) in FA has also designed an open-label study of IFNγ. This study will evaluate safety, tolerability and efficacy in children with FA and is in final stages of IRB review and approval. Enrollment and additional study details are anticipated by late July. FARA is funding this study, and Vidara Pharmaceuticals has generously donated study drug.

HDAC inhibitors – RepliGen recently shared results of the first HDAC inhibitor (HDACi) study in FA, a Phase 1 clinical trial testing the compound RG2833. This trial was designed to study dose and safety. There were four cohorts of subjects who were given single escalating doses of the drug and monitored with various blood and biophysical tests. In the third and fourth cohorts, which were the highest dose levels, the treatment was well tolerated and there was an increase in frataxin mRNA (a measure of frataxin gene expression). These results are significant in that they provide proof of principle that an HDAC inhibitor delivered orally (in a pill) can increase frataxin gene expression measured in blood from subjects. RG2833 is not going to continue forward to further clinical trials with longer term exposure because there are metabolites formed as the compound is broken down in the body that can be harmful. However, over the past two years RepliGen has been developing follow-on compounds that have better access to the central nervous system, better metabolic stability, and increase frataxin similarly to RG2833. A new lead candidate

that meets a very specific set of criteria has been selected, and we look forward to working with RepliGen to get this new candidate advanced as quickly as possible.

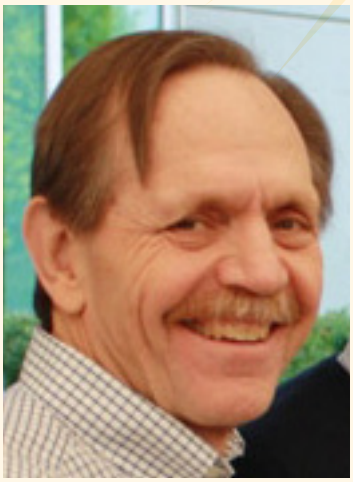
Patients around the world are engaged in clinical trials and moving research forward. This is so important. It continues to amaze me when I hear from other disease groups that their trials are slow to enroll or taking years to complete because of enrollment. We can't afford to have trials fail from lack of participation and time is critical so rapid enrollment makes a difference in months and years to clinical research. Everyone at FARA is proud to be a part of an active and engaged FA community. Everyone finds a way to be involved and contribute and this is what moves treatments across the finish line sooner.

In addition to working on the lead candidates in the pipeline, it is also important to continue to grow drug discovery efforts. FARA has been focused on growing areas of discovery targeting increase, substitution or replacement of frataxin. At the end of last year, Dr. Puccio from INSERM, Strasbourg, France, reported very encouraging and exciting results of a gene therapy approach. She demonstrated that this gene therapy approach could completely prevent and treat the cardiac FA mouse. FARA awarded a grant to Dr. Puccio so that she can continue doing the necessary dose finding animal studies and identifying the best clinical candidate for a gene therapy approach. We have also been assembling and meeting with leaders in the gene therapy community to develop a strategy and path for advancing the program to human trials. We hope to be able to share the details of this strategy later in the year.

My apologies, for not covering all the candidates and early stage research and projects. There is so much going on that I can't report on it all in one article. Please reach out to me with questions at any time – ***jen.farmer@curefa.org***

I will likely respond during those early morning hours.

Please visit ***www.curefa.org/pipeline.html*** for a detailed overview of the FA treatment pipeline with updated information on all of the candidates.



FDA Recognizes FARA Co-Founder and President Ron Bartek

1 of 30 Heroes Changing Lives of Those with Rare Diseases

The FDA Office of Orphan Products Development (OOPD) named Ron Bartek, co-founder and president of the Friedriech's Ataxia Research Alliance (FARA), as one of 30 heroes who have made clinical, research, advocacy and regulatory contributions in the past 30 years to change the lives of those with rare diseases. The heroes were announced in celebration of the 30th anniversary of the enactment of the Orphan Drug Act (ODA), a law passed and designed to facilitate the development and commercialization of drugs to treat rare diseases.

Congratulations, Ron!

The FARA Advocate is brought to you by:
Contributors: Ron Bartek, Kyle Bryant, Felicia DeRosa, Matt Dilorio, Marilyn Downing, Jennifer Farmer, Dr. Bronya Keats, Evelyn Wu, Jamie Young
Editor: Karen Smaalders Cover Design: Crystal Wade
Design/ Layout: Anne Myers

FARA's Research Grant Program Sees Increase in High Quality Proposals

By Bronya Keats, MD

Creating a common grant application deadline in January 2013 has resulted in an increased number of high quality proposals targeting research initiatives of high priority for FARA. Five Letters of Intent (LOIs) were submitted for the Keith Michael Andrus (KMA) Memorial Award for Cardiac Research, eight for the General Research Grant category, 10 for the Phillip Bennett and Kyle Bryant (PB & KB) Translational Research Award, and three for the Bronya J. Keats (BJK) Award for International Collaboration in Research on FA. So far in 2013, FARA has funded the KMA Award and four General Research Grants. Based on the LOIs, four investigators have been invited to submit full applications for the PB & KB Award and two for the BJK Award; these applications are due by July 15th.

In total, FARA is presently providing more than $2.8m in funding for 23 research projects focused on discovering and testing promising new therapeutic compounds (5), accelerating lead candidates through the treatment pipeline to phase III clinical trials (4), developing gene and stem cell therapies (4), improving cell and mouse models to facilitate pre-clinical therapeutic development (4), and advancing understanding of the cardiomyopathy (4) and the molecular pathogenesis (2) of Friedreich's ataxia (FRDA).

FARA-funded research results have been published in many peer-reviewed journal publications. These papers are critical for informing the wider medical and scientific community about advances in FRDA research, which may in turn lead to productive new collaborations. Recent examples include:

• ***A study led by David Lynch and James Rusche, providing support for using frataxin protein levels measured in buccal cells or peripheral blood mononuclear cells (PBMCs) as a biomarker of drug response. Importantly, this study of 41 FRDA patients demonstrated day-to-day stability of frataxin levels, meaning that a 1.5 to 2-fold increase (which is considered to be clinically relevant) would be detectable. (Plasterer H, Deutsch E, Belmonte M, Egan E, Lynch D, Rusche J. PLoS ONE, published online 17 May 2013.)***

• ***A report titled "Increased prevalence of sleep-disordered breathing in Friedreich ataxia" showing that obstructive sleep apnea is much more frequent among individuals with FRDA than in the general population and recommending that those with FRDA undergo regular screening so that treatment can begin immediately if sleep-disordered breathing is diagnosed. (Corben L, Ho M, Copland J, Tai G, Delatycki M. Neurology 81:1-6, May 2013.)***

• ***Research using a transgenic FRDA mouse model demonstrating that bone marrow mesenchymal stem cells (MSCs) have the same effect as those from wild type mice in increasing the survival rate of cultured FRDA mouse dorsal root ganglia cells that had been exposed to stress. This suggests that transplantation of a patient's own MSCs may be a possible therapeutic approach for protecting affected neurons of the dorsal root ganglia (Jones J, Estirado A, Redondo C, Martinez S. PLoS ONE, published online 9 May 2013.)***

A comprehensive list of FARA-funded and other research publications on FRDA is available at: ***http://www.curefa.org/scientific-news.html***

The following newly funded projects cover FARA's priority areas of research- cardiac research, disease (neuronal) models, drug discovery with a focus on repurposed drugs, and advancing candidates in the treatment pipeline.

Recently Awarded FARA Grants

Keith Michael Andrus Memorial Award for Cardiac Research: Mitochondrial Protein Acetylation and Heart Failure in Friedreich's Ataxia

Co-Principal Investigators: Dr. Mark Payne (Indiana University School of Medicine, IA) and Dr. Matthew Hirschey (Duke University School of Medicine, NC)

The goal of this collaborative project is to understand the biochemical basis of heart failure in FRDA. Previously, Dr. Hirschey's research has shown that mitochondrial proteins can be modified (acetylated) and become less active, and that a specific protein (SIRT3) is responsible for restoring their activity to normal, while Dr. Payne has found that mitochondrial proteins from the heart in FRDA are heavily acetylated, which may reduce their ability to use normal fuels (fat) for energy, thus causing heart failure. Together, these findings suggest an important mechanism underlying mitochondrial dysfunction in FRDA, which would explain why FRDA patients develop a fatal hypertrophic cardiomyopathy. Further studies of these findings have the potential to identify a new treatment strategy for FRDA patients by pharmacologically targeting protein acetylation.

Open-label, pilot study of interferon gamma (Actimmune™) for the treatment of Friedreich's ataxia

Principal Investigator: David Lynch (Children's Hospital of Philadelphia, PA)

This CCRN open label pilot study of interferon gamma (IFNγ) will complement and expand on a phase IIa trial of 10 FA patients, aged 18-45, to be conducted by Dr. Roberto Testi in Italy. Dr. Testi's group recently published a study showing that IFNγ raises frataxin levels in FRDA fibroblast cell lines and also in freshly isolated PBMCs from patient samples. The primary objective of the CCRN study is to assess the effect of IFNγ on frataxin expression and protein levels in a heterogenous cohort of 12 children aged 5-18 years with FRDA. Secondary objectives are to assess the drug's effect on neurological outcomes (FARS, performance measures, hearing) and quality of life, as well as its safety and tolerability at the currently approved dose (0.5mL of ACTIMMUNE, 100mcg of interferon gamma-1b per injection, with injections given three times per week).

The pathogenesis of Friedreich cardiomyopathy

Principal Investigator: Arnulf Koeppen (Albany Research Institute, NY)

Cardiomyopathy is the most frequent cause of death in FRDA, and all patients have cardiac dysfunction before developing heart failure or arrhythmia. Since the discovery of iron particles in the hearts of FRDA patients in 1980, this metal has been a focus of FRDA research. Iron-mediated oxidative damage is thought to occur, and recent findings suggest cytosolic rather than mitochondrial iron excess. This project will test two hypotheses: (1) Toxic iron accumulates in FRDA hearts because of an imbalance between import and export of the metal; and (2) Heart disease in FRDA involves "remodeling." A modern technique termed X-ray fluorescence will be used to localize and measure iron and other metals in the hearts of deceased FRDA patients. The extent of remodeling will be determined by specific staining of relevant proteins such as gap junctions. An additional research objective of this project is to confirm that insufficient blood flow through fewer than normal capillary blood vessels is the cause of lack of oxygen and other nutrients in the hearts of patients with FRDA.

Modelling Friedreich ataxia neurodegeneration using induced pluripotent stem cells

Principal Investigator: Mirella Dottori (University of Melbourne, Australia)

FRDA-induced pluripotent stem cells (iPSCs) and their neuronal derivatives retain some of the fundamental molecular and genetic characteristics of this disease, including significantly lower levels of frataxin protein (approximately 30-40%) and GAA repeat instability. However, FRDA iPSC-derived neurons do not show significant susceptibility to cell death or abnormal mitochondrial function. Possible hypotheses for the lack of phenotype is that only specific neuronal populations are sensitive to low frataxin levels and/or frataxin protein levels need to be less than 30% before overt degenerative cellular mechanisms can be identified. These hypotheses are consistent with what is observed in FRDA patients. This project aims to establish an appropriate human FRDA cellular model by genetic modification of the iPSCs to generate a tetracycline-inducible stem cell line that can be induced to decrease frataxin levels at the neuronal stage of differentiation. The relative levels of cell death, neuronal and mitochondrial function will then be measured and compared with controls. These studies will enable the establishment of a human cellular model system of FRDA that can be further utilized to accelerate development of FRDA treatments.

Investigation of diazoxide as a novel frataxin-increasing therapy for Friedreich ataxia

Principal Investigator: Mark Pook (Brunel University, United Kingdom)

The objectives of this project are to determine the therapeutic efficacy of the potassium channel activator, diazoxide, to increase frataxin expression levels and to ameliorate the functional and biochemical disease effects of FRDA. Two mouse models (KIKO and YG8sR) will be used to investigate the effects of both short-term and long-term oral diazoxide treatments. The results of this study may support the rapid progression of an existing drug, for which safety in humans is already established, as a treatment for individuals with FRDA.

Titles and summaries of most of the projects presently funded by FARA are available at: ***www.curefa.org/RPMP/public/pggrantlist.aspx***. Grants awarded by year can be accessed at: ***www.curefa.org/grants-awarded.html*** ●

THE FARA ENERGY BALL

September 5-7, 2013 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.

September 5
Understanding Energy for a Cure Scientific Symposium

September 6
Patron Party

September 7
The FARA Energy Ball with live performance by The Black Honkeys *aka* The Soul Power Review

Thanks to Our Generous Sponsors

PLATINUM SPONSORS
A La Carte Event Pavilion
Lightning Foundation
Standard Meat Company
Liz Smith & Chip Newton
Southern Wine & Spirits
Vidara Therapeutics

DIAMOND SPONSOR
Bill & Vicki Krutzer
Laser Spine Institute
RX Development Doctors Medical
World of Beer

GOLD SPONSORS
Bob and Debbie Basham
Baystage Lighting
Marta Erhard & Thomas DeCotiis
Vic & Lisa Lafita
Doug & Kathy Rothschild
Chris and Sandi Sullivan
USF Health

SILVER SPONSORS
Amalie Oil Company
Paul & Suzanne Gabos
Bill & Charlotte Horne
Bob & Katina Johnston
Lou & Kelly Varsames
Zmithrovitch/ Rattner Family

TABLE SPONSORS
Baldwin Krystyn Sherman Partners
Custom Dessert Solutions
Trenam Kemker
Mitchell & Susie Rice

MEDIA SPONSORS
Broad Reach Television, Inc.
Clear Channel Outdoor
Tampa Bay Times
Tampa Bay Metro Magazine

Design donated by:
Brand Architecture, Inc.

Printing donated by:
Central Florida Press

**As of July 12, 2013*

Presenting Sponors:

MillerCoors™

Paul and Mary Jacobs

The Avery Family Foundation

BLOOMIN' BRANDS

OUTBACK STEAKHOUSE

CARRABBA'S ITALIAN GRILL

BONEFISH GRILL

Fleming's PRIME STEAKHOUSE & WINE BAR

Roy's

The Family Perspective

By Kyle Bryant

When Donna Newman found out about her daughter Natalie's diagnosis in 2010, like most FA parents, she went straight to Google. What she found was not encouraging. She felt helpless, and she didn't know what to do. Then she started finding videos and news stories about Team FARA and their plan to ride across the country to raise awareness for FA research. Seeing others take action gave her great hope, and she knew there was truly something she could do.

Not long after Race Across America, Donna learned that FARA was planning to hold a ride near her community in Dallas, TX. When we emailed her, the wheels were already spinning. Donna was already thinking about ways to get her community and church involved.

"None of us are bike riders, but we knew this was something we could do," she said. She put the word out to her friends, family, and her church and pretty soon the entire town of Mabank, TX was fired up for the ride. They signed up for volunteer positions, recruited the largest team the program had seen up to that point (in bright green tutus!), and launched their fundraising effort. Donna recalls, "We found out that everyone wants to help – we just had to ask."

The church organized a volleyball tournament, a friend held a benefit art class, the local motorcycle club organized a poker run, and the neighborhood had a yard sale. Even Sewell Motors, the employer of Donna's husband, John, got behind the cause as a strong Jersey Sponsor. In the three years that Team Natalie's Hope Riders has been involved with the ride, Donna estimates they have raised $40,000 for research. She adds, "[Each year] it just grows like wildfire."

Natalie appreciates all the effort from her friends, family, and all those involved with the ride. She said, "I think it's awesome how people care so much about finding a cure." Natalie also loves the camaraderie of the FA community at the event, saying, "I love meeting other people who are going through the same thing as me."

Natalie's Team Hope Riders

Due to the efforts of many families like the Newmans, the Ride Ataxia program is off to a strong start in 2013. In Dallas on March 23, despite cold, rainy conditions, 630 cyclists registered and raised over $111,000 for research. In Northern California on June 1, we welcomed 400 riders who raised over $115,000 for research. There are four more rides across the country scheduled this year. You, too, can be part of the movement to take action against FA – to get out and ride, volunteer or fundraise. It is amazing what you can accomplish when you make the decision to act. Together we will cure FA!

Natalie and friends with fellow cyclist Emily Penn

Ride Ataxia Upcoming Events

RA Chicago (Channahon, IL)	July 21
RA Portland (Sauvie Island, OR)	September 21
RA Philly (Blue Bell, PA)	October 13
RA Orlando (Clermont, FL)	November 3

Get all the details at www.rideataxia.org.

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

6TH ANNUAL FRIEDREICH'S ATAXIA SYMPOSIUM

Crowne Plaza
Hotel Valley Forge
260 Mall Blvd,
King of Prussia, PA 19406

Monday, October 14, 2013, 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**

Board of Directors

Bill Alefantis, Director
Broken Plate Group, Founding Member

Paul Avery, Director
President & CEO,
World of Beer

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

Peter Crisp, Director
Vice Chairman,
Rockefeller Financial Services, Inc, retired
General Partner, Venrock Associates, retired

Thomas A. DeCotiis, Director
Founder and Chief Executive
Officer, CorVirtus

Ruth DeWitt, Treasurer/Director
Accountant

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

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

Jennifer L. Good, Director
President and CEO, Trevi Therapeutics

Dr. Holly Hedrick, Director
Surgeon, Children's Hospital
of Philadelphia

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

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

Dr. Steve Klasko, Director
CEO, USF Health Dean,
College of Medicine
University of South Florida

Peter Pitts, Director
Partner & Director, Global Health,
Porter Novelli Senior Fellow,
Center for Medicine in the Public Interest
Former Associate
Commissioner, US Food
and Drug Administration

J. Ed Ramsey, Chairman of the Board
VP, Secretary/ Treasurer, & Co-Owner,
Taylor- Ramsey Corp.
President, Summit Hardwood
VP, BEPCP

Bernard Ravina, MD, MSCE, Director
Medical Director, Translational Neurology,
Biogen Idec, Inc

The Power of Friendship

By Matt Dilorio

Matt DiIorio, who hails from Johnston, RI, was diagnosed with FA on August 4, 1994. He graduated from Bryant University in 2004 with a degree in Business Communications.

In spite of the ups and downs I have had because of FA, I take pride in maintaining a positive and hopeful outlook most of the time. My high school and college experiences were fun-filled and super-packed with numerous activities and escapades. In high school, I managed the varsity basketball team for four years and was thrilled to receive the "Courageous Medal" from the Rhode Island Basketball Coaches' Association my senior year. I attended every social event for the school as well as my class and even went on an awesome senior class trip to California, Arizona, the Grand Canyon, and Las Vegas, Nevada.

Bryant University was my next stop, where I managed the basketball team for two years. Traveling with the team to Albany, New York and San Antonio, Texas allowed me to visit places I may not have seen otherwise. I also joined a fraternity, Delta Chi, so I kept up an active social life. I went to Las Vegas with eight friends who never minded helping me with anything I needed assistance with, such as getting in and out of my manual wheelchair. I also had the perfect internship at WPRI TV Providence in the sports department under Patrick Little. That opportunity was both rewarding and very enjoyable in many ways.

Today my love of sports continues to be a big part of my life. I am an avid fan of the New York Yankees, Boston Bruins, New York Giants, and Providence College Friars. I also enjoy listening to most types of music, especially vocal dance and David Guetta. At Bryant, I started compiling a collection of my favorite songs. I enjoyed playing music that appealed to many for dancing and listening, and I gradually became good enough to play at events and local bars. After college, I DJed for six years and had a great time meeting many wonderful people.

July 12, 2010 was my lucky day! I met Erin O'Neil at a FARA blood draw in nearby Massachusetts. That day Erin became my oldest FA friend. She challenged and motivated me to get involved in FARA. In addition, she told me about "The Race for Grace," a walk, race, roll in Rhode Island that raises awareness and funds for FARA. Although I was unaware of this event at the time, it has now become a very important part of my fight against FA. The event was established in honor of Grace Hopkins, a young girl also living with FA. I was humbled when I learned Grace's family renamed the event "The Race for Matt and Grace." I feel blessed to have a dedicated, loyal, and hard working committee behind me to raise awareness and money for FARA's mission. I am sure Grace would agree.

Matt Dilorio

Meeting Erin that day in July was a turning point for me in many ways. Since then, my family, friends, and I attend many FARA events from Maryland to Philadelphia, to Massachusetts and New Hampshire. This coming October will mark my fourth trip to Philadelphia to attend the FA symposium. The information presented at the symposiums has provided valuable information regarding new medical trials, promising FA research, innovative therapies, and services available to FAers.

What I value most from my living with FA are the genuine friendships that I have made along the way. I am thankful every day for that. I am also grateful knowing that Ron Bartek, Dr. David Lynch, and the entire FARA team are working tirelessly to slow, stop, reverse, and cure Friedreich's ataxia for people like me. I have the utmost respect and affection for these individuals, and I want to thank them for giving me hope. ●

Upcoming Events

July 15, 2013
Purple Summer:
A Benefit Concert for Christina
Philadelphia, PA

July 16, 2013
FARA Give Back With Outback Night
Villa Park, IL

July 17-20, 2013
FA Woodstock 2013
Flying H Ranch – LaPorte, IN

July 20, 2013
Team FARA Blackburn Challenge
Cape Ann, MA

July 21, 2013
Ride Ataxia – rideCHICAGO
Channahon, IL

August 3-4
Team Ryan and Owen Jones -
Change your Latitude Swim (Sitka, AK) and
Pennock Island Challenge (Ketchikan, AK)

August 3, 2013
Team Adirondack -
Adirondack Mountains Hike
Adirondack State Park, NY

August 9, 2013
Welsh Bash in the Backyard
Harrisburg, PA

August 10, 2013
Chris's Fight Car Show
Cave City KY

September 5, 2013
USF Health Friedreich's Ataxia
Scientific Symposium
Tampa, FL

September 7, 2013
The FARA Energy Ball
Tampa, FL

September 8, 2013
Fuzzy Buzzy Golf
Windham, NH

September 9, 2013
Hole Out for a Cure
Peoria, IL

September 13, 2013
FARA Oktoberfest
Leinenkugel Beer Garden
Baltimore, MD

September 19, 2013
Century 21 Golf Tournament
Fontana, CA

September 21, 2013
Ride Ataxia – ridePORTLAND
Sauvie Island, OR

September 21, 2013
Slim's Journey 5K
Warrenton, MO

September 21, 2013
Race for Matt & Grace
Smithfield, RI

September 21
McDonnell Music Concert
Queensbury, NY

September 29, 2013
Team Donovan –
Westchester Triathlon
Rye, NY

October 5-6
Team FARA Run with the Buffaloes -
100 Mile Foot Race
West Yellowstone, MT

October 13, 2013
Ride Ataxia – ridePHILLY
Blue Bell, PA

October 13, 2013
Izzenowzy Rickshaw Derby
Alameda, CA

October 19, 2013
Swing Away At FA
Dawsonville, GA

November 3, 2013
Ride Ataxia – rideORLANDO
Clermont, FL

November 3
Team FARA ING NYC Marathon
NY,NY

November 9, 2013
Stephanie's Hope Holiday Boutique
Santa Clarita, CA

November 9, 2013
Team FARA - Team Mustangs (for Ali)
Savannah Rock and Roll

November 9
Savannah Rock and Roll Marathon
& ½ Marathon
Savannah, GA

Please check the FARA website (www.curefa.org/events.html) for contact information and a full list of events.

CareSync Gives Back

CareSync is a family health record to collect, organize and selectively share your medical records. CareSync connects patients, providers, caregivers, and loved ones with accessible and portable medical records and appointment management tools. Select FARA during the sign-up process and CareSync will donate $10 for each paid subscription (after 3 months) as part of their Giving Back campaign.

www.caresync.com

caresync

Masks Reveal Night of Success!

By Jamie Young

The colorful masks worn at the Night of Mystery Masquerade Ball couldn't hide the excitement and wonderful community support behind the Gordon family, whose event raised more than $30,000 and priceless awareness for FA research!

After she was diagnosed with FA, Anna Gordon and her family felt so welcomed and touched by the support of the FA community that they wanted to give back. Anna, a high school student from West Virginia, combined her new passion for supporting the cause with her dream of attending a Masquerade Ball into a successful fundraiser and community awareness campaign. She personally educated local businesses and organizations about FA and encouraged them to get involved as sponsors. Due to her efforts, the event received special partnering support. Greg Jones, General Manager of the Pepsi Bottling Group in Mineral Wells, was inspired by Anna's genuine passion and focus for helping not just herself, but everyone with FA.

While Anna's mother Melissa, Greg, and other generous committee volunteers focused on the Masquerade Ball details and ticket sales, Anna and her friends focused on awareness, selling "Anna's Army" t-shirts to raise awareness of FA and the Masquerade Ball. Through Anna's courage and willingness to share her story, they sold over 1,000 t-shirts to friends, fellow students, and residents of local and surrounding communities.

Anna's Army T-shirts sold like hotcakes

Anna Gordon

Ten days before the Masquerade Ball, all nervous feelings about reaching their fundraising goal were set aside when tickets and sponsorships for the event completely sold out. On March 23, 2013, the Masquerade Ball featured a fully masked house at the local golf club with great food, music, and fun!

Tips from Melissa and Anna Gordon:

- ***Listen to your kid – If you can combine something they would enjoy within a fundraising opportunity, they will get more involved and feel more comfortable raising awareness for the event.***
- ***Create awareness and the support will come – Approach local business, schools, and use social media (Anna created a Twitter and Facebook event site). This helped to get in-kind donations for the event in addition to attendees and donations.***
- ***Be gracious of everything you receive!***

Luke Leonard leads the pack at the FARA FEST 5k and 1 mi run organized by his parents Tammy and Keith (Virginia Beach, VA)

The Holliday family celebrates with their runner at the finish line

Team Brittany is Getting Fit for FARA in honor of Brittany Perzee (Watseka, IL)

George Perzee (Brittany's dad) weighs in at the end of the 3 month weight loss challenge

GCMS High School staff take a break from waiting tables at D.G. Sullivan's in honor of Emily Young (Gibson City, IL)

Rick, Becky, and Emily Young enjoying another great fundraiser

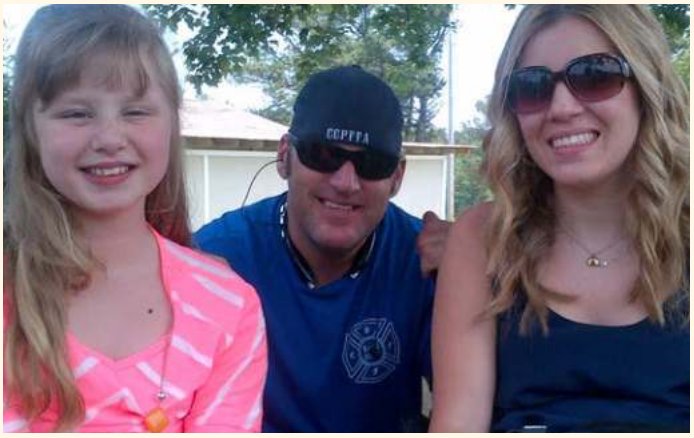
A sea of purple shows the strength of Anna's Army spreading FA awareness (Parkersburg, WV)

The Gordon family presented a Night of Mystery…and fun at their Masquerade Ball!

Kevin, Gavin, and Dawn Lambert enjoy the sun at Anytime Fitness Golf Tournament (Palm Harbor, FL)

Gavin commandeered a golf cart!

The Cobb Professional Firefighters on hand at the 9th annual Laura Beth Golf Tournament (Marietta, GA)



Lovely smiles from Hannah Stacks and Laura Beth Jacquin

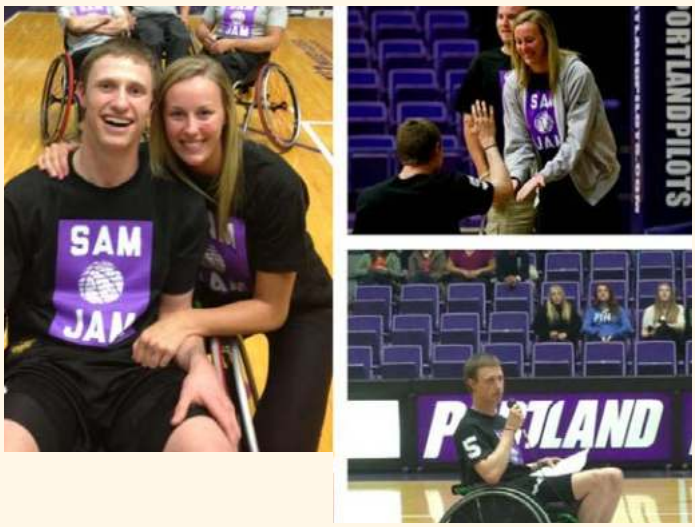
Catherine (left) and Christina (right) with the whole Logan family at the first annual 5k Race for Christina (Newtown, PA)

Christina Logan crosses the finish line!

Matt Dilorio (center) stopped by to enjoy the beautiful spring day and support the 5K Race

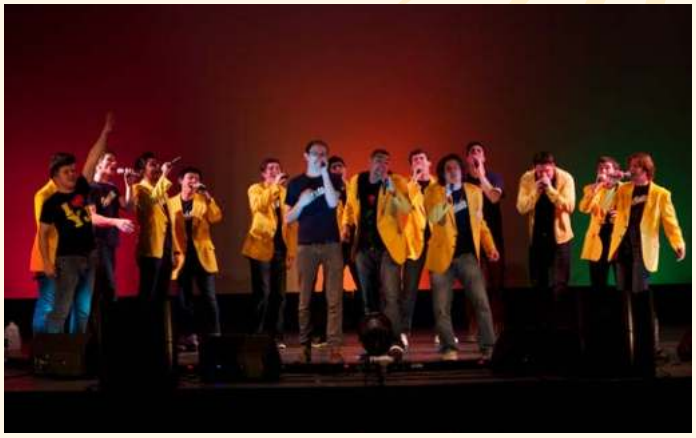
The Penn family and friends braved a chilly Texas morning at the Lone Star Benefit Bash (Flower Mound, TX)

At Pull for a Cure, these men proved they'll do what it takes for a cure by shaving their heads in "Cut for a Cure" to support the Pediatric Cancer Foundation and FARA (Land O Lakes, FL)



Scenes from SamJam 2013 in honor of University of Portland senior Sam Bridgman (Portland, OR)

Sam (center) with his team of All-stars



Galen Dole takes center stage at Concert for a Cure on behalf of his sister Marlise and the Ferrarone Family (Rochester, NY)

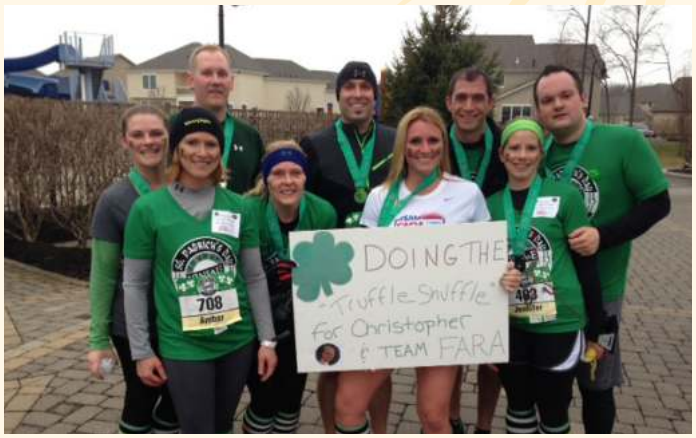
Best friends Sarah Gelbard and Laura Ferrarone get a seranade from the University of Rochester YellowJackets

Kyle Bryant riding strong with Team FARA at Tour de Palm Springs (Palm Springs, CA)

Team FARA riders and volunteers at the Tour de Palms Springs Expo!

The women of Rachel's Runners kicked butt at the American River Parkway Half Marathon (Sacramento, CA)

Breanne Moen is inspired to run by her friend Rachel Gill



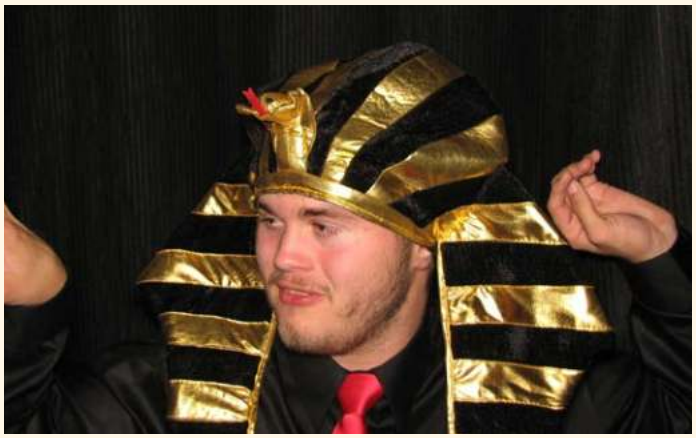
Kelly Alfieri and team show off their socks at the St. Patty's Day 4-miler for Christopher Repass (Powell, OH)

Elizabeth Way makes her Team FARA debut at the Flower City Half Marathon in honor of the Ferrarone Family (Rochester, NY)

Lauren Williams shows no fear on the zip line at Project Adventure Day (Beverly, MA)

Carl Estabrook keeps his cards close and poker face on

Jim, Jamie, and Jennifer Plourde share their love at Spark Hope (Beverly, MA)



Donovan Simpson rolls like an Egyptian

The guests at Izzy's Angels 80s Prom Under the Sea know how to party in honor of the Penston family (Alameda, CA)

Zoe Penston (center) pauses from dancing to strike a pose with friends

RECRUITING STUDY PARTICIPANTS NOW!

The following Clinical Studies are in need of additional participants. Please click on the below links to learn more about each of these studies:

Indiana University- Cardiac Metabolism in Patients with Friedreich's Ataxia
https://www.curefa.org/registry/Documents/Cardiac%20Metabolism%20in%20FRDA.pdf

University of Minnesota- MRI Study
https://www.curefa.org/registry/Documents/flyer_Freidreich_Spinal_Cord_Nov2012.pdf

What is Friedreich's Ataxia?

Friedreich's ataxia (FA) 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.

FARA
533 W Uwchlan Ave
Downingtown, PA 19335

SUMMER 2013

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

www.cureFA.org

FARA Friedreich's Ataxia Research Alliance