WINTER 2014-2015

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

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FARA Friedreich's Ataxia Research Alliance

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President's Message

By Ron Bartek

Dear Friends,

In these president's letters, I have often sung the praises of FA families, the FA community and the FARA family for working so well together and doing the hard work needed to put all the pieces in place to enable progress toward treatments and a cure. You have come to know very well how important it is for all of us to be assembling the resources, infrastructure, relationships and partnerships necessary to accomplish our mission. Over the last couple of months, though, I have been truly amazed and greatly encouraged by the fact that we are now hearing this FARA message being broadcast in a wonderful chorus from all our public and private partners.

That encouraging chorus was heard loud and clear at the 2014 Breakthrough Summit of the National Organization for Rare Disorders (NORD) which brought together over 500 participants October 20-23. Summit speakers included senior leadership from FARA's key partners who sounded as though they were all reading pages from the FARA playbook. The first keynote speaker was Dr. Janet Woodcock, Director of the Center for Drug Evaluation and Review (CDER) at the Food and Drug Administration (FDA). She devoted her presentation to a description of all the ways a rare-disease patient advocacy organization can make a major difference in accelerating therapy development:

1) helping fund basic, discovery science;

2) developing translational research tools such as cell and animal models and bio-repositories of patient samples (e.g., blood, urine, skin cells);

3) developing a natural history database to determine natural progression of the disease, explore biomarkers of the disease, work out clinical outcome measures (endpoints), and design effective clinical trials;

4) helping support a clinical network for patients visits, further developing the natural history database, and conducting clinical trials, and

5) building a patient registry that can be used to recruit clinical trials quickly and effectively.

All this, Dr. Woodcock concluded, will accelerate the movement of discoveries through well conceived translational development, more robust pre-clinical testing and better designed clinical trials so that the new drug approval applications submitted to the FDA will be of higher quality and stand a much better chance of prompt approval.

Now, doesn't all that sound encouragingly familiar? It certainly should, because you, along with FA patients, families, scientists and their supporters everywhere, have been the essential ingredients in our being able together to accomplish every one of those prerequisites. And, Dr. Woodcock's prescription for success was echoed by the other NORD Summit speakers from the government (including FDA Commissioner Margaret Hamburg and key speakers from the National Institutes of Health) and a large number of industry representatives.

The Summit's highpoint came in the lengthy discussion period led by a panel of eight leaders of major pharmaceutical and venture capital companies who echoed this powerful message. As I sat spellbound listening to the eight presentations, I realized that FARA is working closely with five of these eight companies and three of them illustrated their key points by describing FARA programs such as approaches to elevating frataxin protein levels, our natural history database and patient registry, our relationships with the FDA, NIH and industry partners, etc. In speaking with these panelists after the discussion, I learned that their comments about the fine work of FARA and the FA community were made without even knowing FARA representatives were in the room!

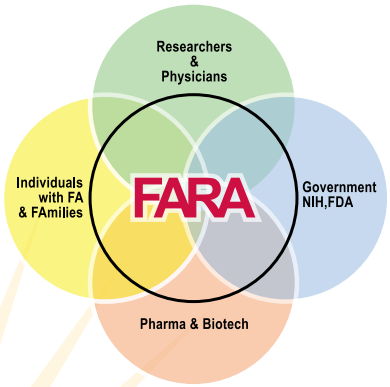
FARA's magic is in the FA patients, families and scientists; the patients who enroll in our registry and are eager to participate in the research, are examined in our clinics by our gifted clinicians and help build our natural history database; our patient families and their friends and supporters who help us assemble the resources needed to advance the science; and the brilliant FA scientists who are devoting their careers to research because they want to make a difference for our patients. This is a magic team that is built to win and, together, we will treat and cure FA.

Advancing The Treatment Pipeline

By Jennifer Farmer

Increased stakeholder engagement has been a key factor in advancing our progress towards treatments. In 2014, there have been more individuals with FA, scientists and physicians, industry partners, government partners, and supporters engaged and involved than ever before, and this has been critical to optimizing our progress.

A recent meeting at Pfizer welcomed the perspective of several stakeholder groups and recognized their importance to advancing research. FARA Representatives and Ambassadors Jack DeWitt, Jamie Plourde and Kyle Bryant shared personal stories and perspectives on living with FA with Pfizer researchers. Dr. David Lynch from the Children's Hospital of Philadelphia provided the medical and scientific overview, and I discussed FARA's role as an advocacy group. The below image illustrates the key groups and how FARA works with them. We believe that success happens in the middle when all the stakeholders are engaged together and working collaboratively. FARA supporters directly influence the size of the FARA circle and the effectiveness of the organization in engaging stakeholders and meeting their individual and collective needs related to advancing treatments.



All stakeholders are critical to move drugs and therapeutic approaches in the pipeline through the discovery, preclinical and clinical testing process towards approved treatments. To that end, additions to our pipeline, reported results and ongoing trials are highlighted here:

Pipeline Additions

• ***Reata's RTA 408,*** is a compound that targets activation of a transcription factor, Nrf2, which should improve mitochondrial function, reduce oxidative stress and protect against diseases involving inflammation. Reata recently received Investigational New Drug (IND) approval from the FDA that will allow them to advance a clinical trial in FA. We expect to begin enrolling a Phase I/II clinical trial in Nov/Dec 2014.

• ***Acetyl L-Carnitine,*** ALCAR, is a naturally occurring compound that is available as a supplement that transports fatty acids to the mitochondria and has an important role in glucose metabolism. Dr. Zesiewicz at the University of South Florida is enrolling subjects in an open label study of ALCAR and evaluating the effect on cardiac and neurological measures in adults with FA. Please contact the USF Ataxia Research Center at 813-974-5909 if you are interested in learning more about this study. Proceeds from the FARA Energy Ball are funding this study.

• ***Incretin analogs*** — Last year FARA awarded the Kyle Bryant Translational Research Award to Drs. Miriam Cnop, Mariana Igoillo-Esteve and Massimo Pandolfo at the Universite Libre de Bruxelles, Brussels, Belgium. This award allowed the team to further evaluate a new treatment approach in FA cell models. The team's initial research was focused on the cause(s) of diabetes in FA. They demonstrated that diabetes develops due to dysfunction and death of pancreatic insulin-producing ß-cells. They also showed that incretin analogs are protective for frataxin-deficient ß-cells. Incretins are gut hormones that control blood sugar levels. Stable hormone analogs have been developed in recent years to treat diabetes. Unexpectedly, they observed that these drugs induce frataxin expression in pancreatic ß-cells. The aim of this preclinical project was to evaluate the potential of incretin analogs to induce frataxin and prevent or improve cell dysfunction. The team tested this in neuronal cells generated from induced pluripotent stem cells from FA patients. Based on the results generated over the past twelve months, the team has launched a small pilot trial of incretin analogs in a few individuals with FA at their University hospital in Belgium. FARA is excited to add incretin analogs to the treatment pipeline, and we look forward to continuing to support this team's progress.

Recently Reported Results

• ***Interferon gamma*** — Dr. Roberto Testi initially reported that interferon gamma (a drug approved for the treatment of two other rare diseases, chronic granulomatous disease and osteopeterosis) increased frataxin in FA cell and animal models and improved neurologic function in an FA animal model. Based on these results Dr. Testi launched a small pilot study in Italy to look at safety, tolerability and dose in adults with FA. Also, Dr. David Lynch, at the Children's Hospital of Philadelphia, launched a small pilot Phase 2a study in children with FA, with support from FARA and Vidara (now Horizon Pharma) which donated the drug for the initial study. The primary purpose was to evaluate safety and tolerability as the drug is known to cause flu-like symptoms as a primary side-effect and to see if there was an increase in frataxin levels. The full results were presented at the CHOP FA Symposium and published in *Acta Neurologica Scandivanica* in October. In summary, while there was no significant change in frataxin levels across the group, individual data in certain tissues (buccal cells) displays a lot of "noise" — levels spiking up and down, which is something that does not normally occur. More intriguing, the clinical data obtained from the study showed a significant improvement in neurological function measured by the Friedreich Ataxia Rating Scale (FARS). It is important to note that this study did not have a control or placebo group, and it was an open label pilot study, so we cannot draw firm conclusions about the efficacy of the drug in treating FA. However, these results are supportive of the need for further investigation. Horizon Pharma, the company that sells Actimmune™ (interferon gamma) in the United States, is committed to moving forward. They are presently in planning stages of what we hope will be a definitive Phase 2/3 study (placebo controlled in a much larger group of individuals with FA). FARA is working closely with Horizon and will continue to keep the community updated as we receive information on the timing and details of the next study.

Ongoing Trials:

• ***Phase 2 EPI-743*** — The EPI-743 study has completed enrollment and the double-blind placebo control portion of the trial. Ongoing is an open-label extension, which was part of the original study design. The study visits for the participants in the open label extension are nearly complete, and we anticipate results in the upcoming months. Edison was granted fast-track status by the FDA earlier this year.

• ***Phase 2 EPO*** — The efficacy Study of Epoetin Alfa in Friedreich Ataxia (FRIEMAX) has completed enrollment and treatment visits. The final stages of monitoring and data query and capture are being completed so that the data can be analyzed. The investigators anticipate results in early 2015 for this FARA-funded study.

• ***Phase 1 Safety and Pharmacology Study of VP20629/OX1 in Adults with FA*** — Enrollment is open and a few more subjects are needed for the final cohorts. If you are interested in learning more about this study or participation, visit **http://www.clinicaltrials.gov/ct2/show/NCT01898884**

The FA treatment pipeline is seeded by FARA grants, which promote discovery science and development of tools, such as animal and cell models needed for validating candidates. Much of this research is carried out by dedicated scientists around the world. Industry (pharma and biotech) partnerships are absolutely essential to drug development and commercialization of new treatments, and we are fortunate to have a highly committed group of partners with proven success in developing new drugs working on almost all our pipeline initiatives. Some of these partners are even involved in the discovery phase of research. The later stages of pipeline advancement rely on FARA supported infrastructure like the Patient Registry and the Collaborative Clinical Research Network which are powered by a dedicated group of clinicians, coordinators, statisticians and patients working together to document the natural history of the disease and to develop improved clinical outcome measures and biomarkers. Clinical trials, the experiments that will tell us if a new drug or therapeutic approach has a beneficial effect, require the collaboration of all our stakeholders: scientists, physicians, patients, and government. We are grateful to all our stakeholders and supporters who allow us to continue to move forward with urgency, confidence and purpose.

FARA's website has detailed summaries on each candidate or program in the pipeline – **curefa.org/pipeline.html** •

FARA's Research Grant Program Continues to Accelerate Progress

By Bronya Keats, PhD

FARA is pleased to announce that Dr. Ed Grabczyk is the recipient of the 2014 Kyle Bryant Translational Research Award. New research grants were also recently awarded to Dr. Andrew Dancis and Dr. Alain Martelli. Each of these projects (described below) clearly demonstrate the importance of FARA funding to advancing our understanding of the mechanism of disease and building on this knowledge to develop promising new therapeutic strategies for FRDA.

Recently Awarded FARA Grants

Kyle Bryant Translational Research Award
Small molecule induced exon skipping of MLH3 to slow repeat expansion in an FRDA mouse model

Principal Investigator: Dr. Ed Grabczyk, Louisiana State University Health Sciences Center, New Orleans

This project will test the hypothesis that increasing GAA•TTC repeat expansion size in disease relevant tissues causes the gradual onset and progression of Friedreich ataxia. Dr. Grabczyk has shown that this continued expansion of GAA•TTC repeats requires transcription through the repeat, then the sequential actions of several DNA mismatch repair (MMR) proteins called MutSbeta (MSH2/MSH3 heterodimer) and MutLgamma (MLH1/MLH3 heterodimer). MutLgamma is the protein complex that cuts the DNA in the repeat to start the expansion. Without the cut, there is no expansion. When FRDA cells are treated with splice-switching oligonucleotides (SSOs), the repeat stops expanding. These SSOs are the same type as those already in human trials for Duchenne muscular dystrophy. This approach has the potential to become a therapeutic reality for FRDA.

A therapeutic strategy for Friedreich's ataxia: Frataxin bypass by enhancing the mitochondrial cysteine desulfurase activity

Principal Investigator: Dr. Andrew Dancis, University of Pennsylvania, Philadelphia

Dr. Dancis proposes a novel therapeutic strategy for FRDA based on the strong evidence that the primary function of frataxin is in Fe-S cluster assembly in mitochondria. Frataxin is thought to be necessary for the formation of an Fe-S cluster intermediate on a scaffold protein called ISCU. The role of frataxin is to stimulate cysteine desulfurase (Nfs1) activity, which is essential for Fe-S cluster synthesis. Dr. Dancis identified a mutant form of Isu1 (the yeast equivalent of ISCU), which enabled normal Fe-S cluster levels in the absence of frataxin, suggesting the possibility of finding compounds that can substitute for frataxin. In this project two libraries will be screened to identify compounds that stimulate Nfs1 activity. These compounds will then be tested for their ability to mimic the effect of the Isu1 mutant that bypasses the need for frataxin in yeast mitochondria. The hope is that the results of this project will point to new drugs or classes of drugs for treating FRDA.

Primary dorsal root ganglia sensory neurons with complete or partial loss of frataxin as models to investigate the neuropathophysiology of Friedreich ataxia

Principal Investigator: Dr. Alain Martelli, Institut de Génétique et de Biologie Moléculaire et Cellulaire (IGBMC), Illkirch, France

Dr. Martelli will develop and characterize new neuronal models of FRDA that will facilitate in-depth investigation of the neuropathophysiology of the disease. Sensory neuron models will be generated by setting up primary cultures of dorsal root ganglia (DRG) sensory neurons from Fxn conditional mice such that Fxn can be either completely deleted or partially deleted using viral vectors expressing human frataxin with a missense mutation. These neuronal models will be characterized particularly with regard to the consequences of frataxin deficiency on mitochondrial metabolic state and transport. The impact of frataxin deficiency on myelin formation and integrity will also be assessed, as well as the role of IRP-1 in cellular iron dysregulation and mitochondrial function. In addition to enabling the elucidation of key processes involved in the neuropathophysiology of FRDA, these DRG sensory neuron models will be useful new tools for testing promising therapeutic compounds.

There is no doubt that FARA funded projects are accelerating progress towards effective therapies for Friedreich's ataxia. During 2014, FARA received 31 letters of intent (LOIs) for our named

and general research awards, reviewed 18 new grant applications and funded 10; we anticipate that at least three more will be approved for funding before the end of the year. As well as the 10 new grants, continuation funds were provided for nine projects. So far FARA has provided a total amount of more than $3.9m in research funding during 2014. This amount includes ongoing support for the Collaborative Clinical Research Network (CCRN) and the Penn Medicine/CHOP Center of Excellence, as well as a grant (cofunded with our FRDA advocacy group partner, GoFAR) to the Jackson Laboratory for the development and characterization of additional mouse models that are needed by the FRDA research community for pre-clinical studies. These models will have larger GAA repeat expansion sizes than those presently available.

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

Recent Publications — FARA-Funded Research

Researchers funded by FARA continue to make important contributions to the scientific literature demonstrating the accelerated progress that is possible because of grants from FARA. Recently published papers based on FARA-funded research include:

- ***A systematic examination of FRDA clinical data*** collected over 12 years from patients attending the Melbourne FRDA clinic. The goal was to explore the natural history of FRDA by analyzing the changes in annual scores of clinical scales (including FARS and ICARS) used to measure disease severity. The study demonstrated that individuals with early disease onset (before 14 years) and large GAA1 repeat size (greater than 669) generally progress more quickly to severe disease, and that FARS and ICARS scores are of most utility as outcome measures for FRDA clinical trials when participants are within 20 years of their age of disease onset. *(Tai G, Corben L, Gurrin L, Yiu E, Churchyard A, Fahey M, Hoare B, Downie S, Delatycki M. Journal of Neurology, Neurosurgery & Psychiatry, published online August 2014.)*

- ***A study of neurons differentiated from FRDA-induced pluripotent stem cells (iPSCs)*** suggesting that relatively low levels of frataxin protein may be adequate for them to function normally. This work also demonstrated that FRDA iPSC - derived neural progenitors integrate into the nervous system and differentiate into neuronal and glial lineages when transplanted in the cerebellar regions of host adult rodents. These findings are encouraging steps towards the potential development of therapies using iPSCs generated from the FRDA patient's skin cells. *(Bird M, Needham K, Frazier A, van Rooijen J, Leung J, Hough S, Denham M, Thornton M, Parish C, Nayagam B, Pera M, Thorburn D, Thompson L, Dottori M. PLoS ONE 9:e101718, July 2014.)*

- ***A detailed cellular, molecular and functional characterization of the GAA repeat expansion FRDA mouse models*** developed by Dr. Mark Pook's laboratory, which will improve the usefulness of these models in investigations of FRDA disease mechanisms and therapy. (Virmouni S, Sandi C, Al-Mahdawi S, Pook M. PLoS ONE 9:e107416, September 2014.) Dr. Pook utilized his YG22 mice in a recent study of the role of MMR protein complexes, specifically MutLalpha (MLH1/PMS2), in intergenerational and somatic GAA repeat expansion and regulation of FXN transcription. This work showed that PMS2 deficiency increases repeat expansion and decreases FXN transcription, whereas loss of MLH1 activity reduces FXN transcription but also reduces repeat expansion. These results add to our knowledge regarding the contributions of MMR system proteins to the molecular phenotype of FRDA. *(Ezzatizadeh V, Sandi C, Sandi M, Virmouni S, Al-Mahdawi S, Pook M. PLoS ONE 9:e100523, June 2014.) Note that Dr. Grabczyk plans to use the YG22 mouse model in his recently funded research project described above.*

- ***A pre-clinical and proof-of-concept study of dyclonine, a drug shown to activate the transcription factor Nrf2, which is approved as a topical anesthetic*** – it is also the active ingredient in Sucrets throat lozenges. Dyclonine increased frataxin levels in FRDA patient cells and tissue from mouse models, and patients using it as an oral rinse showed increased frataxin levels in their buccal cells. Drugs such as dyclonine that activate Nrf2 are potential therapeutics for FRDA. (*Sahdeo S, Scott B, McMackin M, Jasoliya M, Brown B, Wulff H, Perlman S, Pook M, Cortopassi G. Human Molecular Genetics, published online August 2014.)*

• ***An investigation of the histone deacetylase inhibitor HDACi 109*** (RG2833) using FRDA iPSC-derived neurons, and an evaluation of its safety and efficacy in a phase I clinical trial in patients. In FRDA iPS-derived neurons, treatment with RG2833 resulted in the anticipated changes in the epigenetic state of the FXN gene and increased frataxin expression. Similarly, patients in the clinical trial showed a dose-dependent increase in frataxin mRNA in peripheral blood mononuclear cells. RG2833 was well tolerated and no safety concerns were encountered, but laboratory studies indicate that potentially harmful metabolic products accumulate when RG2833 is broken down in the body, which means that it should not be used for long-term treatment. This work provides a promising proof of concept for epigenetic therapy in FRDA, and related compounds with improved metabolic stability are now being developed as candidates for future FRDA clinical studies. *(Soragni E, Miao W, Iudicello M, Jacoby D, De Mercanti S, Clerico M, Longo F, Piga A, Ku S, Campau E, Du J, Penalver P, Rai M, Madara J, Nazor K, O'Connor M, Maximov A, Loring J, Pandolfo M, Durelli L, Gottesfeld J, Rusche J. Annals of Neurology 76:489-508, published online September 2014.)*

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

Grant application deadlines

Grant Type	LOI Deadlines	Application Deadlines	Maximum Budget (in US dollars)
General Research Grant	February 1 July 15	April 1 September 15	$150,000 per year for 1 or 2 years
Keith Michael Andrus Cardiac Research Award	January 15	March 1	$150,000 per year for 1 or 2 years
Kyle Bryant Translational Research Award	May 15	July 15	$250,000 per year for 1 or 2 years
Bronya J. Keats International Research Collaboration Award	May 15	July 15	$200,000 per year for 1 or 2 years

Exceptions to these deadlines are projects that the investigator believes may be of high priority to FARA. An LOI for such a proposal may be submitted at any time during the year. However, the justification for such special consideration must be compelling.

Grant application guidelines are available at:
www.curefa.org/grant.html

Clinical Studies are Critical to Research Advancement!

For more information on the latest clinical research and to learn how you can participate, please visit:

Patient Registry
curefa.org/registry

Collaborative Clinical Research Network
curefa.org/network.html

Clinical Trial Participation
curefa.org/trial.html

Memorials

FARA remembers the friends we have lost, and we thank all who chose to remember a loved one with a donation to FARA. In the past year, we have received over $30,000* in memory of the following individuals:

Aaron Kittel, Byron Rasey, Carlos M. da Silva, Carol DeFilippo, Chelsea Lane, Clint Ziegler, Dan Kirkland Wells, David Allison, Dayle A. Jorgensen, Douglas Spooner, Drew Ducatelli, Eileen Parsons, Eric Christensen, Eugene Lajeunesse, Frank Genalo, G. D. Parrish, Garrett Timbie, George Meusel, Geraldine Schreiber, Gertrude Rouse, Hannah Saunders, Harold Pisciotta, Harrison Charter, Hugh W. Andrus, Jack Reynolds, James L. Taylor, James Loach, James Woods, Jay Caffarra, Jeff Rosenkranz, Jennifer Poplinger, Jerry Sullivan, Jessica Thompson, Joanne Spataro, Joseph A. Harover, Joseph R. Williams, The Fortes – Joseph, John, & Michael, Justin Roake, Karye J. Willard, Kathleen DiPietro, Keith Andrus, Kenneth Dole, Kevin Maher, Kristy Goad Hill, Larry Myers, Margaret Hannemann, Marianne Crisafulli, Marion C. Kimberly, Mary Norman, Matthew Cousins, Mel Caruso, Merrill Rushin, Michael Adinolfi, Michael & Mildred LaFort, Nathan Bell, Nicholas Minichillo, Patricia S. Schamber, Phillip Bennett, Pop-Pop Andresen, Raymond T. Furr, Robert Foster, Robert Fraser, Robert J. F. Borchardt, Robert L. Olander, Robert Petrini, Robin Nunnery, Rudolf Folts, Sara Ferrarone, Suzanna, Thelma Posey, Thomas Barnett, Viriginia Vobejda, William Wagner, and Zac Pavelchak.

To request envelopes to be used for memorials, please contact **FARA at info@cureFA.org.**

*Year to date as of November 11, 2014

Ride Ataxia Shakes a Tail Feather

By Kyle Bryant

At this year's Ride Ataxia in Philadelphia, Team Sean Anderson set an ambitious fundraising goal of $25,000. The team mantra this year was "Anything for FARA," so to help ensure their success, riders sent out an appeal to their friends and family that promised they would ride in chicken suits if they reached their goal. And so their campaign took off — with hilarious Facebook posts as they searched for just the right chicken suits (totally outrageous yet still provide a level of comfort and flexibility needed for their ride). After a bit of trial and error they found just the right suit, but to their surprise the fundraising total kept going up! And up! About a week before the ride, Team Sean Anderson was $800 short of $35,000. So they sent out one last appeal to their friends and family. If they reached $35,000 prior to the ride, they promised to do the Chicken Dance in their chicken suits at the ride (in front of everyone) and film it.

It was an irresistible offer and the dollars rolled in again. In the end, Team Sean Anderson reached $35,773 — all because they will do "anything for FARA."

Here are excerpts from the interview with one of Team Sean Anderson's leaders, Gretchen Anderson:

What motivated you to get started fundraising?

We started out slowly with RA Philly. The first year we participated in the ride, our goal was to raise awareness rather than fundraise (the more people biking, the greater the awareness). We decided after our first year of participation to make this our big fundraiser for a couple of reasons. First, we have only lived back East for 12 years. Virtually all of our family and many of our friends live in California. If we did our own fundraiser in Virginia, only our Virginia friends would be able to participate. Second, with RA, the costs of putting on a fundraiser don't change depending on whether we participate or not. Therefore, everything we raise goes directly to research. This allows all of our friends and family to participate in donating to our team.

Members of Team Sean Anderson (l-r) Sean Anderson, Gretchen Anderson and David Anderson with Kyle Bryant at Ride Ataxia Philadelphia

Our motivation is almost the same today as it was when we first started. We continue to see Sean's progression advance and our friends' children's FA progress, and there is a real urgency to try to find something to at least slow or stop the progression, until a cure is found. On a lighter note, we have a friendly rivalry with Team Laura. We are always ahead at the time of the ride, but they keep sneaking ahead of us after the ride is over! We were determined to stay ahead of them this year. The nice thing is, even if we lose, we win. It means more money to fund a cure.

How do you communicate with your constituents/donors?

We send letters to our previous year's donors. We post on Facebook. We mention RA to friends, family and coworkers. On Nov 1, David is taking a year leave from work to spend more time with Sean. It was announced at Northrop Grumman in July. While some people at David's work were aware of Sean's FA, the seriousness of that diagnosis, and his progression; because of his position in the company, David shared very little with his coworkers, unless they asked. When the leave of absence was announced, that freed David up to talk about it, and he posted our Team Sean Anderson link on all his emails. There was a big response from his coworkers this year.

How does it make you feel to contribute to research?

Sean has been a trial/study participant, and I think we're pretty typical of members of the FAmily in that we are so motivated to beat this thing, before we lose anyone else to FA.

Upcoming Events

In 2014, 2,350 riders attended Ride Ataxia events in 6 locations and raised over $825,000 (gross) in support of FARA's mission. Join us at one or more of these locations in 2015 as we continue to turn the crank toward the cure:

2015 Ride Ataxia Upcoming Events

RA Dallas	April 12
RA NorCal	May 30
RA Chicago	July 19
RA Philadelphia	October 11
RA Orlando	November 1

Get all the details at **www.rideataxia.org.**

rideATAXIA POWERED BY FARA Friedreich's Ataxia Research Alliance

AAi Ataxian Athlete Initiative

Applications for the 2015 Ataxian Athlete Initiative will be accepted from April 1, 2015 to June 1, 2015. The AAI provides, through a competitive grant process, adaptive cycling equipment to people with all types of ataxia who have demonstrated the desire to stay active and healthy despite their disabilities.

For more information visit:
rideataxia.org/aai

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5,4,3,2,1...
Countdown for the Cure

By Felicia DeRosa

It's Countdown for the Cure in the ballroom at A La Carte Event Pavilion

It is easy to be wowed by the numbers of this year's Energy Ball — a sold-out crowd of over 800 attendees, a record-breaking Fund a Cure initiative that raised over $300,000, and an overall event total of $2 million (gross) including in-kind gifts and financial contributions. Even the theme of the event, Countdown for the Cure, showcased numbers on whimsical clocks adorning beautiful centerpieces on every table. Yet the special energy behind those numbers was all about the people — the friends that made up the planning committee, the partners who purchased sponsorship and donated auction items, and the supporters who attended and participated with heartfelt generosity.

The three-day Energy Ball experience started with the Understanding Energy for a Cure symposium hosted by Drs. Zesiewicz and Gooch at the University of South Florida Center for Advanced Medical Learning and Simulation (CAMLS). Speakers included some of our top FA researchers such as Drs. Pandolfo, Puccio, Bidichandani and Gottesfeld, as well as two patient panels. The scientists collaborated on a panel presentation that included an explanation of the genetic origin of FA as well as approaches to therapy at the genetic level. The symposium also included two patient panels — one on clinical trial participation and the other on living with FA. Sam Bridgman, Emily Young, Natchez Hanson and Kyle Bryant were among the panelists who charmed the audience and offered a resilient perspective on the challenges of living with the condition. To view the video from the symposium visit: **http://on.fb.me/1pQQwrh**

The Patron Party sported a laid back Jimmy Buffet theme that transformed a section of the Amalie Arena into "It's 5 O'Clock Somewhere." The party was generously hosted by Honorary Chairs Tod and Tara Leiweke and included delicious fare from Roy's and Bonefish Grill. With an added touch of solidarity, the FARA Team Colors and logo were displayed on all the screens throughout the arena.

The Energy Ball truly lived up to its name — not only as a fun and high energy evening, but one with a sense of magic that results when a community unites behind a common cause. The evening, gracefully hosted by ABC Action News anchor Wendy Ryan, included a meal expertly prepared by the A La Carte Event Pavilion, an inspirational live performance of "The Climb" from The Lockets, and a silent auction with hundreds of items such as memorabilia, spa experiences, sports and concert tickets, and outings to the best of Tampa's dining scene. It also included an exciting live auction that sold exclusive trips to the Adirondacks, Pebble Beach, Colorado and Paris as well as unique experiences to Fly with the Tampa Bay Lightning to an Away Game or be a Fighter Pilot for a Day.

Natchez Hanson high-fives the Tampa Bay Lightning Bug during the cocktail hour

Tom Bradley, Paul Avery and Kyle Bryant present Gavin Lambert with a trophy from the Energy Ball Committee for his efforts raising awareness and funds for FA. Also pictured are Dawn and Kevin Lambert.

Guests "met" families living with FA throughout the evening — whether in posters of the community hanging in the cocktail area, photos on the dining tables or the video of families sharing both the challenge of the disease and the confidence in research — and responded with great generosity during the Fund a Cure Donation call out to help all families living with FA. The formal event concluded but the party continued with a large crowd rocking out to the best of the '80s live performance from The Spazmatics.

Thank you to our devoted planning committee for being ambassadors of the cause to your friends, neighbors and colleagues, and for the time and resources you have invested to make the Ball an ever-growing success. We are also grateful for our friend and colleague, the creative, detail-oriented, multi-tasking Event Coordinator, Ava Forney. And to all of our financial and in-kind supporters, our sincerest gratitude — the Countdown for the Cure truly moves faster because of you.

The FARA Advocate is brought to you by:

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Acclaimed British Pharmacologist Dr. Ian Blair Brings His Wealth of International Experience to FA Research

By David Woods, PhD

Dr. Ian Blair

An article on the front page of the Philadelphia Inquirer on October 19 described asbestos-related mesothelioma and the work being done by the University of Pennsylvania's Center of Excellence in Environmental Toxicology under the direction of Ian A. Blair, PhD.

What's asbestos got to do with Friedreich's ataxia, you might ask. Two things: both the asbestos and the Friedreich's research are like looking for a molecular needle in a haystack — a biomarker, says Blair, who is hunting for that needle in both cases.

An affable ex-Brit, he has served since 1997 as the A.N. Richards Professor in Penn's Department of Systems Pharmacology and Translational Therapeutics. He came to the role following significant appointments at Makerere University in Uganda, at academic institutions in Australia, at Vanderbilt University in Tennessee, and at the Royal Postgraduate Medical School at the University of London.

At the highly prestigious Imperial College of Science and Technology in London under famed tutor Sir Derek Barton he gained his PhD in organic chemistry. He jokes, though, that because he was coming from Uganda, Imperial thought he was a native born African and accepted him under some kind of diversity imperative.

In the early 1970s Uganda was ruled by the bloodthirsty dictator Idi Amin and, if you've seen the movie *The Last King of Scotland,* you'll know that the country was a dangerous place for postcolonial Britons.

Blair's wife of 47 years, Gillian is a forensic psychologist "which makes it easier to deal with me," says her husband. After Gillian gave birth to the couple's first of two daughters, Blair was involved in some harrowing negotiations about visas in order to spirit them out of the country and to safety in Britain. It reminds Ian Blair of another movie, *Casablanca,* that featured a last-minute escape from Morocco.

Blair became involved in FA pretty much by accident. Tom Hamilton, a major donor to the Center of Excellence in Friedreich's Ataxia (a partnership of CHOP, UPenn and FARA), piqued his interest and his desire to contribute. "It brought together a lot of things that I knew something about," he says. "We've developed a biomarker and are looking at ways to measure and isolate frataxin." Now, working with Dr. David Lynch, Blair is exploring metabolic approaches to FA under a subcontract of the Center of Excellence to perform biomarker analyses using novel mass spectrometry-based methodology developed in Blair's lab.

Ian Blair is not simply a boffin, a British term for a person focusing exclusively on technical research and staring through microscopes. The avid golfer climbed Mount Kilimanjaro when he was 18, ran four marathons when he was 50 (he's now 69), rides around in his much loved red Porsche Boxter, and enjoys his six grandchildren, courtesy of his daughters Ester and Emma.

Despite the multiple high-level research and academic positions he's held, including service on the editorial boards of several scientific publications, visiting lectureships in China and Japan, and publications involving 329 peer-reviewed manuscripts, Ian Blair has a rumpled, laid-back manner. He masks his attainments with a jovial, self-deprecating humor. But the plaques on his office wall at Penn attest to a career of huge accomplishment and international recognition.

Much, much more than a boffin, what? •

5th Annual Swing Away at FA: In Honor of Hannah and Austin Stacks

By Jamie Young

Team Photo at Swing Away at FA.

In its 5th year, Swing Away at FA continues to be a grand slam, raising over $42,000 for FA research. The Wiffle Ball Tournament and Family Fun Day, organized by parents Jason and Candy Stacks, provides funds for research and brings together nine FA families.

The idea for Swing Away at FA started with a gathering of friends. They foresaw tough challenges ahead for Hannah and Austin, but knew they needed to act now. After brainstorming ideas and resources, they decided to organize a Wiffle Ball Tournament that soon expanded to include other activities such as face painting, kid's games, an auction, a raffle and of course a delicious southern barbeque lunch. The event takes place outside of Atlanta in Dawsonville, GA, hosted on the beautiful Cabin Fever Farm owned by Mike and Sue Thomas. Once strangers, Mike and Sue are now committed friends of the Stacks who have welcomed the FA community with open arms, embracing the fight to find a cure.

How does a Wiffle Ball Tournament raise $42,000 for research? "Word of mouth — It's been a real grassroots effort," says Candy. "All of our great friends talking about the event and FA with their friends resulted in the growth we have seen over the years. It seems that each year someone in our lives takes a bigger and more active role in organizing and donating. We already have commitments from new supporters for next year."

Over 250 attended this year's Swing Away on October 18th compared to 100 the first year. In addition, fundraising has increased each year for a total of over $185,000 raised over the past five years.

To new families interested in starting a fundraiser, Candy and Jason advise:

"Don't be afraid – do your best to plan, then be willing to handle the challenges as they come…and they will. It is incredibly inspiring to your friends and family when they see you, as parents, fighting back by raising awareness and research dollars. They want to be an integral part to this fight as well – you will be amazed who comes out of the woodwork in support!"

At the start of 2014, FARA set a new goal to raise $1 million through our Grassroots Event Program and titled it "Mission $1 Million". Over 60 grassroots fundraisers answered the call and (at the time of print) raised a record breaking $810,000 (gross) in support of FARA's mission. We anticipate getting very close to our stated goal by year's end. Thank you to the many Grassroots Events Organizers and the countless volunteers, participants and supporters. A complete list of 2014 Grassroots Events can be found at **http://curefa.org/events.html** Together we will cure FA!

The Stacks Family throws out the first pitch.

Liam Dougherty: A Zest for Life and Work

By David Woods, PhD

Liam Dougherty and Virgil

He was diagnosed with FA as a teenager but that hasn't clouded his sunny disposition, punctured his enviable work ethic, or curtailed his evident zest for living.

The one thing that's put the brakes on a bit was not being able to ride his beloved trike for a few weeks. But that's only until he's done wearing a boot to help him heal from a broken foot sustained in a nasty fall.

Nonetheless, speaking from the podium at the recent annual FA symposium, 25-year-old Liam Dougherty said: "I use my trike to commute to school and work; to meet friends for dinner; to get to the gym or grocery store. More than anything I miss the feeling of freedom I get when riding. When I'm on my trike I don't think about atrophy, or neurodegeneration, or heart complications, or life expectancy."

So he zooms all over the city, somewhat to the consternation of his parents, he says.

Liam admits one could see the effects of FA as a list of problems, "but ataxia has taught me to lean into these problems… to fight harder, the bigger the problem seems."

While working on a master's degree in public administration at University of Pennsylvania, Liam is also taking a course in public speaking. However, judging by his oratory at the symposium, where he received a standing ovation, it's clear either that the course is working very well or that he didn't need it in the first place.

Liam gained his BA in liberal arts at St. John's College in Annapolis, the third oldest university in the United States. It's a unique school that emphasizes reading and the classics. There are lectures on Sophocles, Aristotle, Nicomachean Ethics, Dante and Tolstoy. He's even read Tolstoy's epic 1440-page War and Peace. Some of this has clearly stayed with him — he has named his companion dog, a 4-year-old golden retriever, Virgil.

Sitting on a bench on the Penn campus on a sunny day in late October with Virgil at his side, Liam described his extensive and impressive work history. He has worked as a mentor at the Children's Hospital of Philadelphia, providing counseling and emotional support to patients with chronic medical conditions. He served as an intern at the Franklin Institute, and then worked as an assistant teacher in Mighty Writers, working one-on-one with students to encourage creative writing. At Veritas Academy in Phoenix, Arizona, he lectured and led seminars on topics in literature, science, and English. As an intern at WHYY, he wrote and edited articles for printed and online media aimed at younger audiences. "I like to stay busy," he says.

After living with his parents for a couple of years, Liam now has his own apartment in West Philly. He likes to dine out "more than my bank account says I should," he jokes. He's involved in a pharmacologic phase 2 research project for an anti-oxidation medication known as EPI 743 and he's also involved in Ride Ataxia. His girlfriend Brie is studying nursing at Drexel and is hugely supportive without being cloying, he says. He has a fraternal twin brother, a little brother, and an older half brother and half sister.

With his work ethic, personal charm, articulateness and literary interests, Liam seems destined for a career in journalism or teaching. He praises the work and support of FARA and says that he is optimistic but realistic about a cure for FA. The October symposium, attended by some 250 researchers, and FA patients and their families, was a testament to the spirit and hope of the cause… breathing new life into it. Even so, he says, the many presentations from eminent scientists talking about such things as interferon gamma and micro RNA "are completely over my head."

FARA Recognizes Partners, Friends and Allies

By Felicia DeRosa

Tom and Dede Bradley with Sam Bridgman at Ride Ataxia Seattle

In response to the progressive nature of his FA, University of Pennsylvania graduate student Liam Dougherty said:

It is hard not to look at life this way, as a list of growing problems.
But ataxia has taught me to lean in to these problems.
To build muscle where muscle is lost.
To fight harder, the bigger the problem is.
Not only to move in spite of resistance, but because of it.

We celebrate the strength and resilience of Liam's attitude as well as the friends and partners who hold us up as we lean in to the challenges along the path to treatments for FA.

Friend and Ally Award

FARA's efforts on the fundraising and research front continue to gain momentum, thanks to the tireless efforts of countless friends and allies across the country. This year, FARA honors the contributions of Tom and Dede Bradley from Tampa, Florida, with the Friend and Ally Award. Tom and Dede are longtime friends of FARA, offering support at the FARA Energy Ball and other Tampa fundraisers such as the Pull for a Cure Clay Shoot. This year they adopted the Ride Ataxia program at a personal level. In an impressive gesture of loyalty and support, they attended, fundraised for and cycled in five Ride Ataxia events across the country. Even more moving, they arrived early to each event to help mark the routes, spent time befriending FA families, and stayed until the last table was stored and the final garbage bag hauled away. For their home state ride in Orlando, Tom and Dede educated friends and family about FA and brought the largest Ride Ataxia team to date — 110 cyclists! As the FARA staff leaned into organizing a safe and successful Ride Ataxia program, Tom and Dede rolled up their sleeves time and again to hold us up with the sincere support of the best of friends and allies.

General Manager of phl17, Vince Gianinni with Kyle Bryant and Ron Bartek at Ride Ataxia Philly

Media Partner of the Year Award

FARA is proud to honor the exemplary television media support of phl17 and General Manager Vince Giannini with the Media Partner of the Year Award. Vince's longstanding history of promoting and covering FARA events dates back to the Lane family's first Walk to Cure FA in Orange County, CA in 2000, an annual event that generated most of FARA's early grant funding. More recently, Vince and phl17 raised awareness of FA and FARA events with media coverage for Ride Ataxia. Phl17's coverage of Ride Ataxia Philly included a thoughtful multi-part morning show segment the week prior to the event, PSAs to promote the event, an onsite team to support the event, and a photo gallery on their website. Vince, his family, and members of the phl17 staff cycled and fundraised as Team Chelsea to honor the memory of Vince's cousin,

Chelsea Lane. Their efforts have truly raised the profile of Ride Ataxia Philly and helped make it the most successful fundraising location in the nationwide program. In addition, Vince garnered important media support through phl17 affiliates in other Ride markets such as Dallas and Chicago. When FARA leaned in to the need for greater awareness through media, Vince and the phl17 team lifted us up and directed a spotlight on our cause.

Partner of the Year Award

FARA is proud to recognize Catrike, an Orlando-based recumbent trike manufacturer, with the Partner of the Year Award. Catrike values community, and we are honored by their company-wide commitment to the FA community. Catrike is a lead contributor to the Ataxian Athlete Initiative (AAI) — a FARA program that provides access to adaptive cycling equipment through a competitive application process. With Catrike's support, AAI awarded 19 Catrikes since 2009, including one to Liam Dougherty (see page 14- Living with FA). Catrike improves the lives of people in the FA community by helping people with FA remain active. Since Ride Ataxia Orlando started, Catrike and their employees fielded riders and volunteers to support growth for this event and the critical research it funds. Catrike has also raised awareness of FA by promoting Ride Ataxia Orlando to their client community and inviting FARA to speak at Catrike's Annual Owner Rally. FARA leaned in with both a community need for access to adaptive equipment and an organizational need for increased awareness and mission support. Catrike has been a true partner on both of these fronts, providing not only steady support to lean on but also the tools to move forward.

General Manager of Catrike, Mark Egeland with Kyle Bryant at Ride Ataxia Orlando

What is the FARA Ambassador Program?

By Jean Walsh

THE FARA Ambassador PROGRAM

You may have seen one of us at a FARA fundraiser or FA awareness event, read a blog, or received a card from the Ambassador Program. There are 21 of us over the age of 18 with FA who are FARA Ambassadors. We are committed to helping hope come alive by supporting FARA, and shining a spotlight on our vibrant community.

About us:

- *We are volunteers who try to help bridge the gap between researching a cure for and living with FA.*
- *We are positive and supportive, peer representatives for the FA community, raising awareness and funds for FARA.*
- *We are active and visible (mostly we are visible, while some of us are quiet, but strong members) in the FA community with stories online and in the media.*
- *We are focused on community service.*
- *We do not seek credit or acknowledgement.*
- *We KNOW the FA community is supported by many amazing people both in and out of the Ambassador Program; consequently, we aim to highlight everyone's work.*

Our work continues to expand; we:

- *Publish a blog, found on FARA's website;*
- *Send cards to thank folks for hosting a fundraiser, or offer condolences, or celebrate a birthday, etc.;*
- *Speak at events and to the media; and*
- *Are active and visible participants in social media campaigns such as Rare Disease Day and FA Awareness Day.*

We are a service group that serves the FA community and FARA. We are one important part of the effort to live well with FA as we seek a cure! For additional information visit:

curefa.org/ambassadors

Young Investigators Come to Philadelphia

By Evelyn Wu

Young Investigators Yogesh Chutake from University of Oklahoma with Silvia Fortuni and Monica Benini from University of Rome Tor Vergata.

Every October, families from across the country convene in Philadelphia for Ride Ataxia and the FA Symposium hosted by FARA and the Children's Hospital of Philadelphia (CHOP). It's an exciting weekend for the FA community, and this year, special guests joined in on the fun.

Five "young investigators" — graduate students and post-doctoral fellows from around the world — were sponsored by FARA through a competitive application process to present their research at the Symposium and participate in the Ride alongside colleagues from CHOP and University of Pennsylvania (Penn). The awardees were Yogesh Chutake from University of Oklahoma, Silvia Fortuni from University of Rome Tor Vergata in Italy, Joseph (Jiun-I) Lai from Scripps Research Institute in California, Françoise Piguet from INSERM & Université de Strasbourg in France, and Amanda Stram from Indiana University. While the call for young investigators is open to all, these five awardees, who submitted cutting edge and relevant research, are all working on FARA-funded projects.

The Ride/Symposium weekend invited these researchers to connect with our community in addition to discussing their latest research. As they cycled or volunteered at Ride Ataxia, the investigators experienced firsthand the momentum and spirit behind the support for their research. Then at the Symposium's Meet & Greet reception, investigators were welcomed by FARA Ambassadors who were excited to get to know the researchers on a personal level.

"It was highly motivating to see that everyone belonged to the same extended family, with one mission: to find a cure for Friedreich's ataxia," Yogesh Chutake shared about his experience.

To present their research at the Symposium, the young investigators participated in poster sessions, which are an informal way to share research by displaying research data and conclusions graphically. As attendees browse the posters, the researchers can discuss their work and answer any questions. Researchers from CHOP and Penn who also presented groundbreaking work in FA during these sessions included Elisia Clark, Nathaniel Snyder, Qingqing Wang, Kim Lin and Lauren Seyer.

These young investigators are making exciting progress in developing new treatment approaches:

• ***Yogesh Chutake*** is studying ways to reactivate the silenced frataxin gene. He found that the part of the gene that turns it on or off, called the promoter, is silenced in people with FA, and that the degree it is shut off depends on the length of the GAA expansion. He is also studying whether RG2833 (an HDAC inhibitor treatment approach) may be increasing transcription by turning on the FXN promoter.

• ***Silvia Fortuni*** is working on increasing frataxin levels by blocking its degradation process in patient cell models. She found and is now developing a better compound that protects frataxin from degrading, increasing the amount of frataxin in cells and rescuing some mitochondrial defects caused by low frataxin. She also believes she has identified the previously unknown enzyme that degrades frataxin, which may present a new target for treatment.

• ***Joseph (Jiun-I) Lai*** has developed a way to genetically remove the expanded GAA repeats from patient DNA using the helper dependent adenoviral vector (HdAV) method. Using this technique, he is working on investigating the interaction between the GAA repeats and other genes using microarrays. This approach may also have the potential for gene therapy in the future.

• ***Françoise Piguet*** is working on two projects. She seeks to develop better sensory neuron cell models and the tools needed to study mitochondrial activity in these cells. Her ultimate goal is gene therapy for neurological symptoms in FA; she is currently developing a related study in mice.

• ***Amanda Stram*** is studying the cause of heart failure in FA, specifically a protein called sirtuin 3 (SIRT3) that decreases with frataxin loss. The combined loss of these proteins disrupts heart metabolism and causes damage in two steps: first when the heart's relaxing function declines, then the contraction function. She plans to study the effects of protein replacement and a drug that may assist SIRT3 in heart-specific mouse models. By learning more about the mechanism of heart failure in FA, she hopes to identify potential preventative and treatment strategies.

Read the original abstracts from all the posters presented at the 7th Annual FA Symposium held on October 13, 2014:
http://curefa.org/_pdf/CHOPposter2014.pdf

Developing Biomarkers for FA

By Jane Larkindale, PhD

Biomarkers are key tools in drug development that accelerate progress, but so far we have very few that have been developed for Friedreich's Ataxia. FARA determined that this was an area where we could impact the rate of therapy development.

A biomarker is something we can objectively measure that reflects changes in a biological process. For example, blood samples donated from patients led to the development of an assay for frataxin, so that we can measure the protein in patient's blood and see if an experimental drug has increased the levels. This helps us know if the drug is doing what it was expected to do. Unfortunately it does not tell us if the patient will improve, as blood is not an affected tissue.

A similar marker in affected tissues would be even more valuable. A different type of biomarker could identify patients on the verge of cardiac issues, telling us which set of patients should be included in a new trial. Another might help show how quickly the disease was progressing, informing us about how long a trial should take, and when an intervention should be tested. In a best possible scenario a biomarker that changes rapidly as the disease progresses can be used as a surrogate endpoint, reducing the length of trials. In FA, we have only limited data on any of these. At FARA, we feel that lack of quality biomarkers is a barrier to progress that we need to address.

FARA partnered with the National Center for Advancing Translational Sciences (NCATS, part of the National Institutes of Health) to organize a meeting to determine what studies should be done to develop high quality biomarkers for FA. Approximately 50 people attended the meeting, including experts in FA, experts in biomarker development and companies with an interest in developing therapies for FA. The meeting was set up to encourage discussion, and there was lively debate between the participants. Many possibilities were suggested — some which had never been considered in FA previously, others where significant data exist-

ed. Discussion ranged from biochemistry to electrophysiology to imaging studies to gait analysis, while always remaining focused on what could be measured in important tissues to FA, and what those measurements would mean. Experts from outside of the FA world suggested technologies not previously studied in FA, while FA experts compared and contrasted with known FA data to determine which would have the highest probability of success.

The meeting has provided FARA with a priority list of potential biomarkers that might be relevant to FA. We now need to start both pilot studies to see if biomarkers not previously considered in FA might be feasible, and longer-term studies to see how markers with some evidence might change over time in patients. FARA will partner with companies working in these areas to fund and run these studies, and the resulting data will be available to everyone in the community. Development and validation of biomarkers should both accelerate progress in developing new therapies, and make the field even more attractive to drug developers.

RECRUITING STUDY PARTICIPANTS NOW!

The following Clinical Study is in need of additional participants. Please click on the link below to learn more about the study:

University of Minnesota- MRI Study

https://www.curefa.org/registry/Documents/flyer_Freidreich_Spinal_Cord_Nov2012.pdf

Team Tara for FARA rode to Cure FA at Ride Ataxia Orlando (Clermont, FL)

Colette, Celise, & Emma traveled from Sydney, Australia to run the NYC Marathon in honor of Emma's brother Nick (New York, NY)

The 9th Annual Stephanie's Hope Holiday Boutique brought people together to shop for a cure in honor of Stephanie Magness (Santa Clarita, CA)

The Price Family appreciates all the runners and supporters on Team Mustangs who rocked the Savannah Full & Half Marathon in honor of Alison Price (Savannah, GA)

John Acton, Jack DeWitt's grandfather, celebrated his 80th birthday to benefit FARA (Howell, MI)

The group tackles a team-building exercise at Day 1 of FA Adventure Days, organized by Jean Walsh and Project Adventure (Beverly, MA - Photo by Woody Barr)

Friends work together to hoist Felicia DeRosa in the air at Day 2 of FA Adventure Days (Photo by Woody Barr)

Hosted by the Hook Family, FA Woodstock is a fun retreat complete with camping, swimming, fishing, yoga, live music, arts & crafts and fireworks (LaPorte, IN)

Team Emily represented at Ride Ataxia Chicago (Channahon, IL)

Thank you Amanda Parker and Outback Steakhouse in Lumberton, NC for organizing the Aussie 5K for FARA

The group strikes a pose after a great day of riding at Ride Ataxia Seattle (Redmond, WA)

Friends and neighbors enjoyed the Welsh family's 8th Annual Bash in the Backyard in honor of Brendan (Harrisburg, PA)

In its 5th year, Mike Delsignore & Team Adirondack braved a 4-day backpacking trip in Montana in honor of Dylan McDonnell

Gabe Larios (pictured) & Dave Norman participated in the Louisville Ironman in memory of Dave's wife Mary and in honor of the Coppi Family

Dave and Chelsea Mengyan held their wedding in honor of their friend Jack DeWitt (Clarkston, MI)

The DeWitt family enjoyed a great day of golf at their "Weapons of Grass Destruction Tour" (DeWitt, MI)

Friends of Jerod and the Laird family hosted another successful Reverse Draw dinner (Bakersfield, CA)

Fuzzy Buzzy golfers show up in style for their friend Erin O'Neil (Windham, NH)

The McDonnells put on a rockin' show each year (Photo by Upstate Boys Film - www.upstateboys.com)

Dylan is serenaded by his dad Dave at the 7th Annual McDonnell Music Festival (Queensbury, NY - Photo by Upstate Boys Film)

Grace Hopkins and Matt Dilorio celebrate with friends after the 5th Annual Race for Matt & Grace 5K (Providence, RI)

Ron Bartek rolls Donovan Simpson across the finish at the 10th Annual Team Donovan effort at the Westchester Triathlon (Rye, NY)

Anna Gordon was all smiles at the Rocky Mountain Bird & Birdie golf tournament and clay shoot to benefit FARA (Denver, CO)

The 3rd Annual Slim's Journey 5k Run/2.5k Walk had another successful year in honor of Justin "Slim" Myers (Warrenton, MO)

Roman and his friend Tristan enjoy the great food at Joeseppi's Italian Restaurant at the Roman's Regiment fundraiser (Tacoma, WA)

Friends Carly Stempel and Angelina & Samantha Ritschel are ready to take on Ride Ataxia Philly

Joey Mullaney and Matt Blumenthal, along with the Caruso/Bode family, organized Team FARA Ride Connecticut with support from SigEp Fraternity (Quinnipiac University - Hamden, CT)

Sam Bode prepares for the 10 mile trail at Team FARA Ride CT

FARA
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WINTER 2014-2015

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

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