

SUMMER 2015

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance



THIS ISSUE:

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

FARA | Friedrich's
Ataxia
Research
Alliance

CUREFA.ORG



Advocacy to Advance Therapeutic Development

By Ron Bartek

Dear friends,

I sure hope the summer of 2015 has been treating you and your families well. With all the exciting Research and Development (R&D) progress reported in this issue, I wanted to tell you a bit about the ways in which you and your FARA family are working to advocate for a legislative, regulatory, governmental and commercial environment in which our concerted R&D efforts to treat and cure FA have the best chance to succeed.

First, a word about FARA's general approach to advocacy. FARA advocates on behalf of and in front of its stakeholders and partners — the FA patient community, academic investigators, industry partners, government agencies such as the National Institutes of Health (NIH) and the Food and Drug Administration (FDA), and patient advocacy organizations — in pursuit of policies and decisions intended to advance therapeutic development.

At the center of FARA's current advocacy efforts is draft legislation called the 21st Century Cures Act. This draft act created and advanced by the House Energy and Commerce Committee contains provisions that, if enacted into law, could have significantly beneficial impact on the discovery, development, and delivery of treatments and cures to the rare disease patient community. Two such provisions would result in much needed budget increases for our key Government partners — \$550 million more for the FDA and an additional \$14.5 billion over 10 years for the NIH. Such increases would help FA academic investigators and industry partners secure NIH support and make it easier for the FDA to obtain the personnel and resources it needs to promptly and effectively review FA clinical developments.

In addition to these proposed budgetary boosts, the draft Act contains some provisions of particular interest to the FA community. One of these is the Pediatric Rare Disease Priority Review Voucher Program, which was established by legislation enacted in 2012. This Program was intended as a pilot to test the feasibility

of incentivizing therapy development for pediatric rare diseases by awarding a priority review voucher to companies that develop and get FDA approval of a novel treatment. The company can then sell the voucher to another company interested in a 6-month priority review of its application for approval of a therapy for a common disease — a review that would otherwise require 10 months or more. The third priority review voucher issued under this Program sold in May 2015 for a total of \$245 million. The draft 21st Century Cures Act contains a provision to reauthorize the pilot Priority Review Voucher Program, which would otherwise terminate in March 2016. FARA has helped lead the effort to support the reauthorization of the Program and to define a "Pediatric Rare Disease" in such a way that FA is eligible.

As always, FARA is helping build and participate in effective coalitions that present a chorus of patient voices to advance these important advocacy objectives. These coalitions frequently include our colleagues at the National Organization for Rare Disorders (NORD), where I continue to be an active member of both the Board of Directors and Advocacy Committee. FARA also collaborates energetically, especially in regards to the NIH and FDA, with the excellent umbrella organizations Research!America and the Alliance for a Stronger FDA. For example, read about FARA as the featured advocacy organization on the FDA Alliance's website <http://strengthenfda.org/members/member-spotlight/>. We also look for every opportunity to work in concert with our other advocacy organization partners such as the Muscular Dystrophy Association (MDA) and with our industry partners committed to developing FA therapies.

Of course, our most important stakeholder and coalition advocacy partner is YOU — the FA patient community. We will continue to seek your active involvement at key moments in important advocacy activities. For example, we will no doubt turn to you to ask that you write letters to your senators and congressional representatives at key points in the legislative process as the NIH and FDA budgets and the 21st Century Cures Act make their way toward final passage in the Senate and House. As you know, we are all in this together and it is together that we will treat and cure FA.

Thank you and warm regards,
Ron



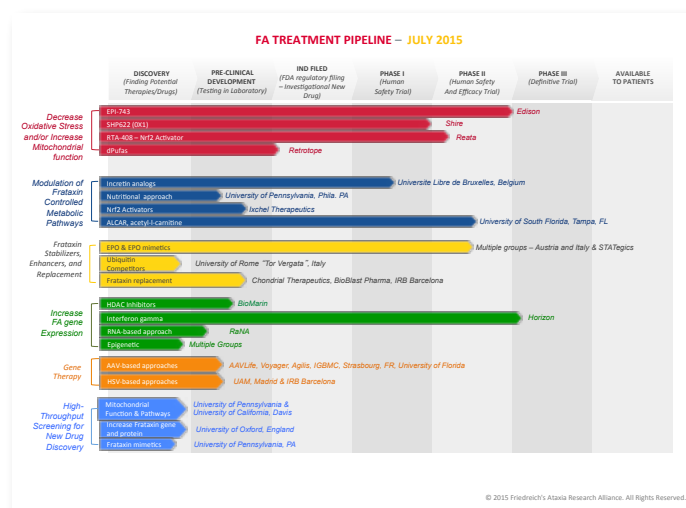
Treatment Pipeline Progress and New Clinical Trials

By Jennifer Farmer

Have you seen FARA's new website at www.curefa.org?

We just finished a redesign and content management upgrade that we are really excited to share with you. We hope the improvements will help you find information more easily.

As part of the website makeover we also had our pipeline image redesigned.



You no longer need to tilt your head 90 degrees to read the bars! This new design allows us room for future growth of new mechanisms of action and lead candidates and makes it easier to track progress.

Down the left hand side you'll find the categories of candidates based on mechanisms or approaches to treatment. Each candidate advances from the left to the right with various milestones identified across the top, from early research or discovery phase through clinical research phases to approved treatments.

FARA is working with pharmaceutical and biotech companies and academic researchers who are advancing these lead candidates in a variety of ways.

- FARA facilitates introductions and develops collaborations between companies interested in working on FA and academic researchers and clinicians with expertise in the disease.

- FARA's grant program has provided funding to 16 of the 19 candidates or drug screening programs listed.
- FARA's grant program has funded research for new cell and animal models and other research tools that are used to test drugs in the pipeline.
- FARA provides funding and leadership for the Collaborative Clinical Research Network in FA which conducts research on the natural history, clinical measures, biomarkers, and quality of life, all of which are necessary to inform and expedite clinical trial design and execution.
- FARA's Patient Registry is used to recruit individuals for clinical trials. When we are able to have individuals quickly identified for research studies we can shorten the time line of the study by months.
- FARA advocates for policy initiatives that provide greater awareness, financial resources or other support for research, drug development and regulatory flexibility and efficiencies for FA and other rare diseases.

As of July 1, 2015 we have three clinical trials and many clinical research studies actively recruiting and enrolling – read more below and click on the titles to link to the FARA website for more details, study site flyers and contact information.

- **Horizon's Actimmune Phase 3 Trial (STEADFAST)** – The STEADFAST study opened enrollment in June 2015 at Children's Hospital of Philadelphia and University of Iowa. This study is enrolling 90 individuals with Friedreich's ataxia (FA) ages 10-25 years who are able to walk 25 feet with or without assistance. Additional study sites include University of Florida which opened enrollment on July 9th and University of California Los Angeles which is expected to open enrollment later in July. Also, in April, Horizon Pharma plc announced that they were granted Fast Track Designation from the FDA for ACTIMMUNE® (interferon gamma-1b) in the treatment of FA. This Fast Track Designation may be very helpful in shortening the timeline to approval of the drug for FA if the results of the clinical trial are favorable.
- **Reata Phase 2 Trial for RTA 408 (MOXIe)** – Enrolling individuals with FA ages 16-40 years who are able to perform 10-15 minutes of exercise on a recumbent exercise bike. We are pleased to announce that 16 patients have been enrolled to date in the MOXIe study. Reata is opening two additional eight patient cohorts to test additional doses between 20mg – 40mg of study drug. A third cohort of eight patients will be available at the following sites: University of Florida, The Ohio State University,

University of South Florida, and Emory University. Once eight patients are enrolled in Cohort 3, an additional eight patients may be enrolled in Cohort 4 later this summer. Additional sites, which include University of California Los Angeles and Children's Hospital of Philadelphia, will be available later this summer for Cohort 4.

- ***Cardiovascular Effects of Acetyl-L-Carnitine*** – Enrolling individuals with FA 18-80 years, at the University of South Florida, Tampa, FL
- ***University of Minnesota MRI Study*** – Enrolling individuals with FA ages 10 and over who have very early symptoms and/or are newly diagnosed or have had pre-symptomatic diagnosis at the University of Minnesota, Minneapolis, MN
- ***Weill Cornell Study of Cardiomyopathy*** – Enrolling individuals with FA 18-30 years at Weill Cornell, New York, NY

We are also happy to report that enrollment and the active study for the Phase I VP20629/SHP622 was completed at the end of June. No further subjects are needed. The sites and the company are now working to lock the database. Thank you to all who participated in this study.

As a community, by fundraising and participating in research, you are changing the profile of the treatment pipeline to add more bars and advance the existing ones. We are honored to steward the dollars you raise to fund good scientific research, and we are grateful for your time spent traveling to participate in clinical research. Thank you for your commitment on all fronts of FARA's mission. Together we will treat and cure FA! ●

FARA Welcomes Erin Goerss



I am very excited to be part of the FARA team as the Gift Processor and Administrative Assistant. In this position, I am responsible for gift processing, supporting other members of the FARA staff, and making sure the day-to-day operations of the office run smoothly. I received my M.A. from Temple University and worked for five years in education administration at a charter school.

I may have already met you over the phone or email, and I look forward to working with all of you towards our goals of finding treatments and a cure for FA. I feel so grateful for this opportunity, and I know I couldn't have found a more wonderful community to work with!

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.

Research Grants Look At New Therapies

By Bronya Keats, Ph.D.

FARA is always pleased when outstanding researchers who have not previously been involved in Friedreich's ataxia research express interest in applying their expertise to projects that advance our understanding of Friedreich's ataxia (FRDA) and suggest novel therapeutic approaches. Thus, we were triply delighted to award funding recently to Dr. Hugo Bellen, Dr. David Corey and Dr. Zhen Yan for the following exciting projects, and we welcome them to the FRDA research community:

- ***The role of iron accumulation and increased lipid synthesis in the pathogenesis of Friedreich's ataxia***

P.I.: Dr. Hugo Bellen, Baylor College of Medicine, Houston

Dr. Bellen, Distinguished Service Professor at Baylor College of Medicine and a Howard Hughes Medical Institute investigator, is a pioneer researcher in the development of fruit flies, *Drosophila melanogaster*, as a powerful model for studying human neurodegenerative disorders. He is a leader in the field, well-known for his generosity in freely providing these models to the research community. He found a mutant fruit fly with some symptoms similar to FRDA. As the fly ages its neurons slowly die, but if the human frataxin gene is expressed, the fly lives a normal lifespan and nerve death is reduced. As has been shown in some human FRDA tissues, iron accumulates in many tissues in the fly, and metabolism is impaired. Interestingly, treatment with a compound called myriocin, which affects a class of lipids, slows the neuronal loss. Dr. Bellen's data shows links between iron deposits, impaired metabolism, the balance of lipids in the cell, and the death of the neurons. This project aims to investigate the mechanism of neurodegeneration in fly frataxin mutants, which could be highly significant for the development of new therapeutic strategies for FRDA.

- ***Activation of frataxin expression by duplex RNA***

P.I.: Dr. David Corey, University of Texas Southwestern Medical Center, Dallas

Dr. Corey is a Professor of Pharmacology and a world leader in the identification of a specific type of drug, oligonucleotides, which may be useful for drug development for FRDA. Oligonucleotides are small sections of DNA or RNA that can be coded

for various purposes. Dr. Corey proposes to use specific RNAs he designed to recognize the expanded GAA repeat region within the frataxin gene. He hypothesizes that these molecules will bind to the repeat sequence in patient cells and interfere with other molecules believed to be attracted to the site that prevent frataxin transcription. By binding his molecules to the repeat sequence, he hopes to prevent the cell from switching off the frataxin gene so more frataxin can be produced and the cell can stay healthy. Early data in FRDA cells shows the duplex RNA can increase frataxin protein to levels similar to cells from unaffected individuals. In this project, Dr. Corey will optimize these compounds and provide insight into its mechanism. If successful, this will lead to a novel strategy for increasing frataxin in FRDA patients.

- ***Exercise impacts on mitochondria and muscle function in Friedreich's ataxia***

P.I.: Dr. Zhen Yan, University of Virginia

Dr. Yan is the Director of the Center for Skeletal Muscle Research at the Robert M. Berne Cardiovascular Research Center, and an expert in exercise physiology and the pathobiology of muscle. Over the past decade, his research has focused on the impact of exercise training on muscle mitochondria using a mouse model of exercise. Mitochondria are typically described as the powerhouse of the cell, and their function is impaired in FRDA patients. Exercise training is a potent means to improve mitochondrial quality in multiple tissue types in unaffected individuals, and therefore could be a valuable tool to help us understand what is going wrong in FRDA mitochondria and, potentially, how to improve FRDA mitochondrial function. Dr. Yan has recently developed a novel reporter gene to measure the health of millions of mitochondria in live animals. In this project, he plans to conduct the first exercise study using this state-of-the-art technology to assess the impact of exercise on mitochondrial health in a mouse model of FRDA. The findings from this study have the potential to advance understanding, management and treatment of FRDA.

- ***Keith Michael Andrus Memorial Award for Cardiac Research***

Our congratulations to Dr. Alice Pébay from the University of Melbourne, the recipient of the 2015 FARA Keith Michael Andrus Memorial Award for Cardiac Research. For the past eight years, Dr. Pébay has applied her expertise in stem cell research to the development and characterization of induced pluripotent stem cells (iPSCs) derived from FRDA patients. iPSCs are cells made from any human cells (usually skin cells) that exhibit embryonic-like

Continued

characteristics and can be turned into any type of cell. In particular, she has worked out how to generate large numbers of iPSC-derived cardiac (heart) cells. The focus of her project, "Screening of phenotypic abnormalities in Friedreich ataxia induced pluripotent stem cell-derived cardiomyocytes," is to use this technology to generate and study cardiac cells derived from patient cells and to characterize them. She will then develop a drug-screening platform looking to reverse the pathologies that she can measure in the cells, thus allowing the potential for identification of new treatments for FRDA, specifically the cardiac issues associated with FRDA.

The FARA Grant Process

FARA is presently supporting 24 research projects, all of which underwent rigorous peer-review by at least two reviewers before being approved for funding by the FARA Board of Directors. Our grant reviewers are essential to this process; we sincerely thank all of you who devote time and effort to reviewing grant applications submitted to FARA.

Grant application instructions, including deadlines for LOIs and full applications, as well as grants awarded by year are available at curefa.org/grant.

New FA Research Publications

Over the past 6 months FARA-funding has resulted in 18 peer-reviewed publications. These include:

The successful correction of FRDA patient lymphoblasts, fibroblasts, and iPSC-derived neurons by excision of the GAA repeat expansion using a zinc finger nuclease. The corrected cells had increased frataxin expression, aconitase activity, and ATP levels. This work is a critical step towards the development of cell replacement therapy. (Li Y, Polak U, Bhalla A, Rozwadowska N, Butler J, Lynch D, Dent S, Napierala M. *Molecular Therapy*, published online 11 March 2015)

The identification of a set of ubiquitin-competing molecules that increase mature frataxin levels and aconitase activity in FRDA cells. These small molecules prevent frataxin precursor degradation by the ubiquitin/proteasome system and the results of this study support their potential role as a novel therapeutic strategy. (Rufini A, Cavallo F, Condo I, Fortuni S, De Martino G, Incani O, Di Venere A, Benini M, Massaro DS, Arcuri G, Serio D, Malisan F, Testi R. *Neurobiology of Disease* 75:91-99, March 2015)

The visualization of frataxin expression in single FRDA patient cells showing that the presence of the GAA repeat expansion changes the position of the FXN gene in the nucleus, which may be linked to the decrease in the number of FXN mRNA molecules and their lower rate of synthesis. This study advances our understanding of the mechanism by which the GAA expansion represses frataxin levels. (Silva A, Brown J, Buckle V, Wade-Martins R, Lufino M. *Human Molecular Genetics* 24:3457-71, June 2015)

A comprehensive list of FARA-funded and other research publications on FRDA is provided at: curefa.org/scientific-news ●

FARA Welcomes Ann Musheno



In February 2015, I joined FARA as the Grassroots Event Coordinator. My main role is to help our wonderful FAmilies plan and execute their own fundraising events across the country as part of our Mission 1 Million program! Additionally, I assist the Energy Ball Committee and FARA Advocacy Committee with their ongoing efforts to raise funds and awareness for FA.

I recently graduated from Northeastern University in Boston with a B.A. in Journalism. During my time at Northeastern, I participated in a co-op program where I worked in event planning at several different nonprofits, most recently the National Alliance on Mental Illness of Massachusetts.

Though I had no prior personal connection to FA, I am honored to be able to join such a passionate, dedicated community!

Dr. Kimberly Lin: Young Patients Seek Honesty and Trust in Medical Encounters

By David Woods, Ph.D.

As an assistant professor of pediatrics at the Children's Hospital of Philadelphia (CHOP), and as the mother of 8-year-old Emily; 5-year-old Katie; and 2-year-old Nate, Dr. Kimberly Lin can stake a strong claim to knowing everything about kids from both a professional and a maternal standpoint.

She is quick to point out, though, that children are pretty smart patients. "They'll feel you out," she says. "They're suspicious at first; they're not sure when they can trust you."

To approach them in a nonthreatening way, Dr. Lin has abandoned her white coat and seeks to gain her young patients' trust and confidence through being honest and straightforward. "If they're scared about an injection, tell them up front that it might hurt just a little."

Of course, Kim, as she's widely known even to her patients, brings something special and of inestimable value to her charges. To begin with, at 38, she's a bit closer to their age than many of the physicians they'll encounter; but perhaps more important, she brings a warm smile and a hearty laugh to the doctor-patient relationship. "It's a privilege to work with young people," she says.

Dr. Lin did her internship and residency at the University of Michigan, Ann Arbor, where she also became chief resident in pediatrics. Following that, she became a fellow in pediatric cardiology at CHOP where she was also a fellow in pediatric cardiomyopathy and heart transplantation.

Today, she's an assistant professor of pediatrics at CHOP and attending physician at that hospital's Department of Pediatrics, division of cardiology.

Beyond the day-to-day clinical work, Kim serves as a peer reviewer for Journal of Heart and Lung Transplantation and Cardiology in the Young, and she has lectured and published widely in her specialty. Among the 15 or so co-authored abstracts she's produced, one seems especially consonant with her views about the doctor-patient relationship: "Pediatric Trials Are Not Small Adult Trials."



Dr. Kimberly Lin

Dr. Lin has won awards for excellence in clinical skills, for outstanding clinical performance and the American Heart Association's Women in Cardiology trainee award for excellence. She is a member of the International Society for Heart and Lung Transplantation, the American Academy of Pediatrics, the American College of Cardiology and the American Heart Association.

As a member of a heart transplant team, Dr. Lin says that most of her work comes before and after, rather than during, surgery. She also selects patients who are suitable for a transplant, which is not a cure, she explains – it's trading one medical condition for another. A lot of the pre-and postoperative input involves working with parents and relatives of the patient. Counseling. Caring. Again, that's where her warmth and empathy serve her well.

She chose to work in cardiology, she says, because "It's exhilarating. I've always loved it because it consists of both the art and the science of medicine."

Dr. Lin's involvement in Friedreich's ataxia in general, and FARA in particular, came about, "because I was in the right place at the right time," she says. "I went to a couple of FA meetings and got to know people in the Alliance."

FARA's executive director Jen Farmer notes that "Dr Lin's keen sensitivity to her patients' experiences not only informs her clinical care but also her research questions, so that all of her work stems from improving the quality and length of life." ●

To learn more about Dr. Lin's Cardiac Care in FA Program,
[click here.](#)

To learn more about Dr. Lin's Cardiac Research Studies in FA,
click below:

[**Exercise in Friedreich's Ataxia**](#)
[**Cardiac MRI and Biomarkers in Friedreich's Ataxia**](#)

Largest International Ataxia Research Conference Held in UK

By Jane Larkindale, Ph.D.

On March 25-28, 2015, FARA partnered with three other ataxia research organizations to host the International Ataxia Research Conference (IARC) in Windsor, UK. More than 340 people from around the world attended the meeting, including representatives of more than 16 pharma and biotech companies. The meeting covered Friedreich's ataxia and other ataxias; sessions focused on all stages of research from basic understanding of the diseases to clinical trials. New data was presented, causing the corridors to buzz with researchers talking, sharing ideas and forming new collaborations. This was the largest ataxia research meeting ever held —both in terms of numbers of attendees and the number of presentations.

The main scientific sessions were very well attended, covering a broad range of topics that maintained interest and prompted a lot of questions from the audience. Early sessions addressed the disease cause and molecular mechanisms, allowing basic scientists to delve deeply into what is going wrong and why. This is essential to understanding how interventions can slow disease progression—and resulted in collaborative discussions about the value of different pathways, how drugs in development might affect different pathways, and how to develop new therapies. This moved smoothly into sessions on evolving therapeutics and then to clinical trials, where the more basic scientists asked mechanistic questions that kept drug development companies on their toes and ensured these key questions are answered in early development.

Other sessions included such important issues as how to design clinical trials and how to develop new and better animal and cell models for testing therapeutics. These sessions once again stimulated great interactions between scientists, as basic scientists proposed new ways of looking at the disease, drug development companies made a case for the tools they need, and others suggested optimal ways to test new drugs.

Several patients spoke during the meeting about their experiences with FA. How could researchers fail to be inspired after hearing from Helen Kearney and her path to a Paralympic gold medal in



Kyle Bryant with conference attendees following his speech at the Oxford Town Hall.

equestrian, or the poetry of Clodagh Clerkin? Kyle Bryant's keynote address in the Oxford town hall received a standing ovation. Scientists traveled home not just exhausted from the introduction of so many new ideas and concepts, not just excited by new experiments to try and new collaborations to get underway, but also inspired by the ataxia community, which has worked together to get to where we are, and will continue to support the path to treatments and a cure. ●

Join The FARA Patient Registry!

Get notified about new trials and help advance FA research!

www.curefa.net/registry



THE FARA ENERGY BALL

September 17
Understanding Energy
for a Cure
Scientific Symposium

September 18
Patron Party

September 19
Energy Ball

Sept. 17, 18, and 19, 2015
in Tampa, Florida.

To benefit FARA and
USF Ataxia Research Center

Book your tickets online now.

www.curefa.org/EnergyBall

Donations in lieu of attendance are
gratefully accepted.

Thanks to Our Generous Sponsors

PLATINUM SPONSORS

A La Carte Event Pavilion
Avion Park Westshore
Horizon Pharmaceuticals
Paul & Mary Jacobs
Lightning Foundation
Standard Meat Company
Southern Crafted Homes
Southern Wine & Spirits
World of Beer

SILVER SPONSORS

Anna Maria Oyster Bar
Rick & Dixie Berman
Ms. Terry Lea Pepin
DTZ
I Plan It, Inc.
Trinchero Family Estates
Tower Radiology Centers
USF Foundation
Vic & Lisa Lafita

DIAMOND SPONSOR

Jabil

GOLD SPONSORS

Bay Stage Live
Chondrial Therapeutics
Corporate Creations
Laser Spine Institute
PDQ
The Ritschel Family
Chris & Sandi Sullivan
USF Health
Vanguard Protex Global

MEDIA SPONSORS

Broad Reach Television, Inc.
Tampa Bay Times
Tampa Bay Metro Magazine
Design donated by:
Brand Architecture, Inc.
Printing donated by:
Central Florida Press

*As of July 7, 2015

Presenting Sponsors:



The Avery Family
Foundation



Spotlight on rideATAXIA NorCal

By Jaime Young

“The ride is where we all come together to support a much greater cause and that being to one day find a cure...Being at the ride is like having a second family who understands and is always there.”

– Sean Mazeres, after the sixth rideATAXIA NorCal

rideATAXIA NorCal brought together over 400 participants and volunteers to Davis, CA on May 30, raising more than \$110,000. The ride had 5-, 6-, 19-, 40- and 60-mile route options to allow participants of all abilities to achieve their goals. While the ride continues to be a successful and necessary source of funding for FA research, it also serves FAmilies in others ways. Sean Mazeres and his family have been a part of all six rideATAXIA NorCal events since Sean was diagnosed in 2009 at age 16. Sean says, “I was looking for anything to pull me up after being diagnosed the very first year of the ride. I needed to know that everything was okay and that I wasn’t alone in the process of healing. I found that at the ride! I found friends and support! I would really encourage new families to come out and join.”

While Sean continues to fundraise and recruit teams over 25 people, his family and team are also involved in another very important aspect of the ride...the food! His dad, Greg and UC Davis Dining Services, along with their many generous vendors help stock the NorCal rest stops with delicious food to fuel the riders to the finish line. At the rest stops you can find a variety of goodies such as granola bars, baked goods, fresh hummus, veggies alongside tuna sandwiches and mini hot dogs, and a personal favorite of rideATAXIA Director Kyle Bryant, hummus-tuna sandwiches. For the past three years, Sean has ridden a recumbent trike and joined his team out on the course. “Don’t be afraid to get out there and ask for help,” he says. “Seeing the support from my personal community has given me a positive thought that everything is achievable and that people do want to help out and make a difference. My team shows me this every time that they ride alongside me and reach out to help bring funds in for research.”



Sean Mazeres getting ready to ride in NorCal

“If I could try to sum up the ride in one word it would be resourceful. Resourceful is defined as having the ability to find quick and clever ways to overcome difficulties. We all bring different things to the table. We all are looking for any way to bring hope and make a difference to come together and overcome one important obstacle, not having a cure. But we are doing everything in our power to bring awareness and raise enough funds to keep going to research so that someday here in the near future we will find a cure.”

– Sean Mazeres

The ride, like the cure, is a puzzle and every FAmily, cyclist, volunteer, and donor make up a crucial piece to make the ride a success. Thank you to Sean and the many rideATAXIA supporters around the country for doing your part. ●

Please Join us for an upcoming ride:

RA Chicago	July 19
RA Philadelphia	October 11
RA Orlando	November 1

Get all the details at www.curefa.org/ride-ataxia

Ataxian Athlete Initiative Announces 2015 Recipients

By Kyle Bryant

The Ataxian Athlete Initiative (AAI) provides adaptive cycling equipment to people with any type of ataxia who have demonstrated the desire to stay active and healthy despite their disabilities. This year, the AAI was sponsored by our partners at Catrike, The FA Project, The Texas Irish Foundation, and a contribution in memory of Gladys Lacativo.

FARA is pleased to announce the 2015 AAI grant recipients: Lee Bailey of Melbourne Australia, Marcos Alfonso Garcia Campo of Barcelona, Spain, Mandy Davis of Clinton, MO, Tiffany Gambill of Bridgewater, MA., Sean Mazeres of Woodland, CA, Kimberly Welch of Bridgeville, PA, and Billie Wells of Southfield, MI.

AAI grants are administered through a competitive application process. Applicants research the most appropriate adaptive cycling equipment to suit their abilities and describe how such equipment would help them to reach their fitness goals and improve their quality of life.

The AAI has provided equipment for 33 individuals since 2009. Visit curefa.org/aa1 for more information and to view photos of past recipients. The next application cycle begins in Spring 2016.

For those looking to get started in adaptive cycling, please see "A Guide to Beginning Your Search For Adaptive Cycling Equipment." curefa.org/rideataxia-blog/a-guide-to-beginning-search-for •



FARA Store

For yourself, your family and friends, or to have customized FARA items at fundraising events, visit the FARA store!

www.cureFAstore.com.

FARA caps, polo and t-shirts, wristbands, etc.

Board of Directors

Bill Alefantis, Director
Broken Plate Group,
Founding Member

Thomas Hamilton, Director
President and CEO,
Construction Forms Inc.

Paul Avery, Chairman of the Board
President and CEO,
World of Beer

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

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

Dr. Steve Klasko, Director
President,
Thomas Jefferson University
President & CEO, TJUH System

Dr. Sanjay Bidichandani,
Scientific Director
Section Head of Genetics, University of
Oklahoma College of Medicine
Department of Pediatrics

Geoffrey Levitt, Director
Senior Vice President and
Associate General Counsel, Regulatory
& Policy, Pfizer, Inc

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

Dr. James McArthur, PhD, Director
Chief Scientific Officer Cydan
Development Inc.

Tony Plohoros, Director
Principal at 6 Degrass PR

Ruth DeWitt, Treasurer/Director
Accountant

J. Ed Ramsey, Director
Co-Owner & VP, Taylor-Ramsey Corp.-
Retired

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

VP, BEPCO- Timber & Land
Development Company

Vincent R. Giannini, Director
Senior Vice President/General Manager
WPHL-TV, Philadelphia
WDCW-TV, Washington D.C.

Bernard Ravina, MD, MSCE,
Scientific Director
VP of Clinical Development, Voyager
Therapeutics Inc.

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

Pat Ritschel, Director
Former Director, New Business
Development at MECS

The FARA Advocate is brought to you by:

*Contributors: Ron Bartek, Kyle Bryant, Clodagh Clerkin,
Felicia DeRosa, Jennifer Farmer, Erin Goerss, Dr. Bronya Keats,
Jane Larkindale, PhD, Ann Musheno, David Woods, PhD,
Evelyn Wu, Jamie Young*

Editor: Karen Smaalders

Cover Design: Crystal Wade

Design/ Layout: Anne Myers

Graphic Support: Lawrence Phillips

Light

By Clodagh Clerkin

The last rays of sunlight filter through the grey, the beams become diffuse as they strobe the forest floor like parting spectators as they vacate at the end of the show.

Leaves reveal their speckled reluctance to submit to the day, at first they sway defiantly like the wildebeest that exhausts its reprieve as the pride fold in and shatter its earthly illusions. The bark is desolate as its majestic stance becomes reduced to an eery shadow casting an unease in all who dwell below.

Some take slumber and retreat into the folded and creviced margins, but others emerge from their murky and silent wait.

The leaves have become all but resolved to their bitter fate as they turn their gaze to the earth below, dropping in tandem like the beaten army retreating to their verges.

The night is long and the canopy masks the stars and the moon, so that all hint of light and beauty is obscured by its enveloping prow.

It is not sudden as dawn decides to loom. At first it tentatively caresses the edges, feeling cautiously for any hidden danger, but soon it is feeling brave as it senses those that lie asunder, urging for appeasement and a chance to feel those cascading rays warm against their fringes.

As it washes in, it bathes the forest floor in a smooth coat of renewed purpose. The birds sing with their steady stream of amorous intention. The leaves lift their gaze to the sky once more and spread themselves thick and wide, ready to absorb the sun's energy and nourish every ounce of their being.

The bark stands tall once more, rooted firmly within the ground. It will not sway nor will it fall. It will hold its stature and announce its strength and nobility to all.



Clodagh Clerkin, a patient representative for Ataxia Ireland, was inspired to write the following poem at the 2015 International Ataxia Research Conference. Here, she recites "Light" at the conference closing.

Clodagh, 29, lives in Ireland. She was diagnosed with FA when she was 24. Her eldest brother and her cousin also have the condition. She likes to write about everything, from science and nature to philosophy and poetry. She recently finished her first novel, a crime fiction, and is currently seeking publication. Her other great passion is photography. Her dogs are often the unsuspecting subjects of her photos. As well as spending time with them, she likes to travel and is currently planning a trip to Asia for the end of the year. Her two weaknesses in life are her "muscles and coffee." ●

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.net/registry

Collaborative Clinical Research Network
curefa.org/network

Clinical Trial Participation
curefa.org/active-clinical-trials

Hundreds Don Leis and Flip Flops for FARA Fest

By Ann Musheno

It was a sight to behold as hundreds of people ran, walked and rolled down the boardwalk at Virginia Beach clad in grass skirts, coconut bras and flower leis as part of the third annual FARA Fest Lei Day 5K. The Jimmy Buffet-themed event on April 18, 2015 featured a timed 5K and a Flip Flop 1-Mile Walk/Run. The tailgate beach party offered free food and drinks, music, a frozen concoction stand and a parrot head best dressed competition. The event was a huge success, attracting over 800 attendees and raising more than \$37,000 to support FA research.

The first FARA Fest was conceived by a group of FA parents, including Tammy Leonard, whose 11-year-old son Luke lives with FA. Tammy says the group wanted a fundraiser that would serve as an “opportunity for the community to come together, have a fun day and learn about FA.”

Their idea took off, with the number of attendees tripling since last year. Tammy attributes this growth to joining up with the families of Alyx Holliday (featured on the cover of *The Advocate*) and Stacey Gentry, who also live with FA. The Jimmy Buffet theme also helped create a broader recognition for the event outside of FA families and their immediate networks.

While the event appeals to those outside of the FA community, the true purpose of the event is not lost. “We have done a really good job telling our participants year by year what FARA Fest re-



FARA fest was a fun day for everyone (Virginia Beach, VA)

ally benefits and what research progress has been made,” Tammy says. This year, FARA staff member Jamie Young spoke at the event to share news of such advances as the biomarker consortium and two new clinical trials.

In addition to educating the public about FA and the research underway, FARA Fest also provides a chance to celebrate people living with FA overcoming some of the obstacles the disease poses. Tammy’s favorite part of the event is seeing her son, Luke, participate in the Flip Flop 1 Mile. She gets goose bumps seeing her son cross the finish line as the crowd cheers him on, recognizing the extra effort it took for him to finish the race.

For families new to fundraising for FARA, Tammy advises: “Once you get an idea in your head, don’t be afraid to go off of that... It’s only a bad idea if you don’t pursue it. The worst thing that can happen is nothing.”

FARA Fest is one of over 60 grassroots fundraisers held by families across the country each year. In 2014, FARA set an ambitious goal to raise \$1 million in the year, called Mission 1 Million. That goal was exceeded and the program is expected to once again surpass \$1 million this year. To learn more about grassroots events occurring across the country or how you can host your own grassroots fundraiser, visit curefa.org/grassroots. Together we will cure FA! ●



Luke Leonard and FARA Fest’s mascot (Virginia Beach, VA)

Grassroots Fundraising



Golfers take a break at the Anytime Fitness Golf Tournament (Palm Harbor, FL)



New Cavern Productions hosted Concert for Christina: A Tribute to The Beatles in honor of their friend Christina Logan (Philadelphia, PA)



Jeff Morgan hugs his cousin, Erin O'Neil, after completing The Boston Marathon as part of Team FARA (Boston, MA)



Brianna Mahoney completes The Boston Marathon as part of Team FARA (Boston, MA)



Shelby Pelletier takes a break from running The Boston Marathon to give Kaela Mullaney a hug (Boston, MA)



Christin Haun poses with the Brown Family at BA Cure FA, a fundraiser featuring a historical walk of Broken Arrow's beautiful downtown rose district. (Broken Arrow, OK)

Grassroots Fundraising



Staff from FARA and CHOP catch a photo with the Logans, Dilorios and Welshes at the 3rd Annual Mother's Day Race for Christina 5K (Richboro, PA)



The Logan Family hosted another successful Mother's Day Race for Christina 5K (Richboro, PA)



Michelle Huebner's son shares a few words with guests at Cure Us: An Evening to Benefit the Friedrich's Ataxia Research Alliance (Portland, OR)



The crowd enjoyed fine food, company, and auctions at Cure Us (Portland, OR)



Erin O'Neil put on another successful fundraiser at Flatbread Pizza Company (Bedford, MA)



At the patient conference at the University of Iowa, Emily Young answered questions about her life with FA and how she overcomes challenges (Iowa City, IA)

Grassroots Fundraising



Karen Reid, Marilyn Kiess, and Andrea Kiess take a break to chat at the patient conference at the University of Iowa (Iowa City, IA)



Team Alison Price shows their medals after competing in the Best Damn Race (Safety Harbor, FL)



Jill Gould held a garage sale to raise funds for FARA (Largo, FL)



Team Alison Price after the Gasparilla Distance Classic (Tampa, FL)



Marilena Wilkinson ran a 210km ultra-marathon along Sri Lanka's Elephant Trail as part of Team FARA (Sigiriya Rock, Sri Lanka)



The Dilorio family kicked off a season of fundraising, leading up to the Race for Matt & Grace, with a party at McShawn's Pub (Cranston, RI)

Grassroots Fundraising



Team FARA participated in Penn Medicine's Million Dollar Bike Ride for Rare Disease Research (Philadelphia, PA)



Members of Team FARA before running the Rock 'n' roll Half Marathon (Nashville, TN)



Attendees enjoyed their day at Tampa Bay Sporting Clays with the Pull for a Cure event (Tampa, FL)



The crowd at Parkersburg South High School's Purple Out pep rally in honor of Anna Gordon (Parkersburg, WV)



The Gordon Family enjoyed a carriage ride to their Masquerade to Cure FA (Parkersburg, WV)



rideATAXIA director Kyle Bryant gives a speech to motivate cyclists at rideAtaxia NorCal (Davis, CA)



Cyclists at the start of rideAtaxia Dallas (Denton, TX)



Rare Disease Day events were held at state capitols around the country to advocate for those living with rare diseases. Here, attendees at Pennsylvania's Rare Disease Day hold up signs reminding us that there are 15,000 people worldwide who live with FA (Harrisburg, PA)



Karen Ryan and Karen O'Brien put on another successful New Jersey Seaside Luncheon complete with raffles and lunch catered by Carrabba's (Seaside, NJ)



Attendees at the New Jersey Seaside Luncheon had the opportunity to win an assortment of raffle items organized by Maureen Sweeny (Seaside, NJ)

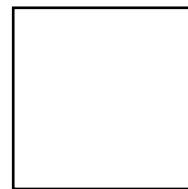


John Ryan enjoyed this year's Seaside Luncheon with friends and family (Seaside, NJ)



At a dinner fundraiser to benefit her rideAtaxia NorCal team, Megan McCandless poses with event planners Joe Gough and Terry Barragan, and karaoke DJs Margie and Cort Stratfull (Rancho Cordova, CA)

FARA
533 W Uwchlan Ave
Downingtown, PA 19335



SUMMER 2015

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance



FARA | Friedreich's
Ataxia
Research
Alliance

CUREFA.ORG