WINTER 2016 - 2017

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

This Issue:

FARA Friedreich's Ataxia Research Alliance

CUREFA.ORG

President's Message

By Ron Bartek

Dear friends,

As many of us feel an autumn chill in the air and some have even seen early snowflakes, very promising FA clinical trials continue to push forward. Two of them are in late stages – Horizon's phase 3 trial of Actimmune (interferon gamma) is nearing completion and Reata's RTA-408 is moving smartly through a phase-2 trial. Retrotope also just finished its first-in-human study. As you know, a number of other important therapeutic approaches are in preclinical studies and advancing toward clinical trials. Among these are such exciting strategies as the HDAC inhibitors at BioMarin, TAT-Frataxin at Chondrial Therapeutics and gene therapies advanced by several excellent companies and universities.

Because of the rising number and accelerating pace of these FA clinical programs, FARA has stepped up its efforts to build on its already excellent relationships with the Food and Drug Administration (FDA). For example, we have secured FDA agreement that FARA will organize and conduct an "Externally Led Patient–Focused Drug Development Meeting" on June 2, 2017 near FDA headquarters in Silver Spring, MD. At this meeting, key FDA personnel will hear the "patient's voice" about which symptoms most affect the activities and quality of daily living and which therapeutic benefits would be most important to FA patients. Over the first few months of 2017, FARA will work with the FA community to prepare for this half-day meeting. So, please save the date for June 2 to join our FDA colleagues, a few FA scientists and a good number of patients and patient-family representatives, with a larger number of patients and families participating online. Online participation will be facilitated via an interactive system that will encourage participants to enter, simultaneously with the live participants, their views and thoughts on the same topics discussed on location. Following the meeting, FARA will prepare and publish a "Patients' Voice" report compiling all the live and online input. The meeting and the subsequent report will help instruct FDA personnel in their consideration of all FA proposals and submissions.

FARA has also secured FDA agreement for a much smaller, shorter meeting with a particular segment of the FDA – the Center for Biologics Evaluation and Research (CBER). To date, FARA has spent the vast majority of its FDA-focused time working with the Center for Drug Evaluation and Research (CDER) because all the FA clinical trials, so far, have involved testing of therapeutic drugs that, consequently, have been considered by CDER. Gene-therapy research, however, is within the purview of CBER, so FARA has begun the process of extending its excellent FDA relationships and educational efforts to CBER reviewers and administrators. We have been communicating with these CBER personnel, some of whom already know a considerable amount about FA, to plan a short, focused meeting during the first quarter of 2017. The meeting is to include key FA scientists as well as the companies and universities already committed to FA gene-therapy programs.

Of course, these meetings are in addition to the many opportunities FARA has had to participate with our pharma partners in pre-trial meetings with FDA reviewers, the many times FARA has been invited to speak at FDA symposia and the many FDA personnel who have participated actively in FARA conferences. All of these activities have built and nurtured our excellent relationships with the FDA reviewers and administrators who now know FA better, understand our science and the needs of our patients better, and will be far better prepared to give our therapy-development programs the prompt, effective review and consideration we seek and the FDA strives to provide. In sum, these meetings and relationships give FA clinical research and therapy development our very best shot at approved therapies to treat and cure FA.

Warm, appreciative regards,

Ron

Growth of FARA's Research and Initiatives Expand Impact

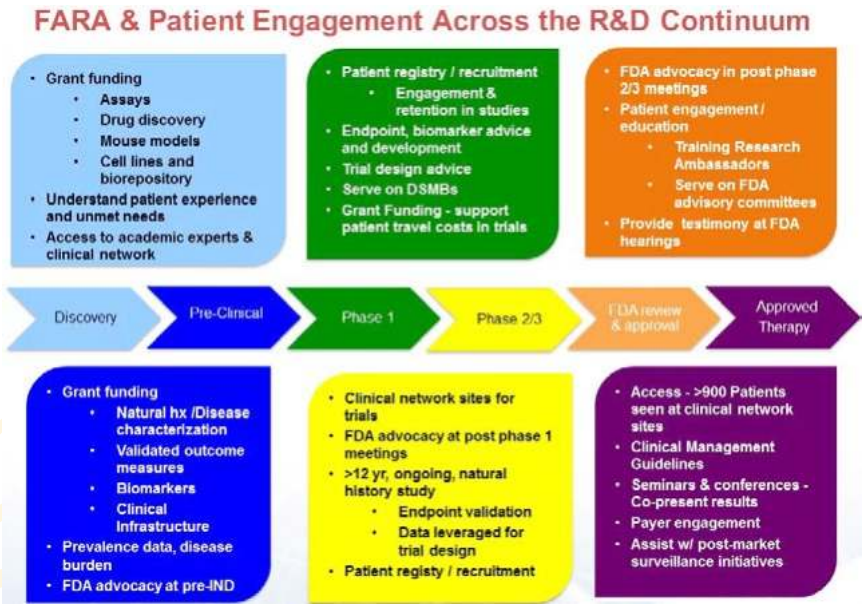
By Jennifer Farmer

I started part time with FARA 11 years ago, charged with managing a young $600,000 grant program, initiating a patient registry and launching a clinical network. While these programs are still firmly in place in service of FARA's mission, the scope of each has grown tremendously as evidenced by the number of pharmaceutical partners, therapies in development and patients in trials. This is a direct result of both a passionate scientific and patient community and the generous support of countless donors.

FARA funds and facilitates research, but what does that look like today? The diagram below offers some perspective. Through the lens of this continuum, we can see how FARA's work supports treatment development at various stages. Some activities bring new treatment candidates to the pipeline like assays and models for drug discovery. Some activities help advance existing candidates through the pipeline such as patient registry recruitment and trial design advice. All demonstrate that FARA and the FA community wear a number of different hats to advance potential treatments for FA.

FARA's largest investment ($4-5 million annually), the scientific grant program, resides on the early part of the continuum. Academic scientists or small biotech companies submit grant applications to FARA for funding. These applications are peer reviewed and grants are awarded to projects with meritorious science that are aligned with FARA's priorities such as drug discovery, understanding the underlying mechanism of the disease, and cardiac research. (See page 5 for a chart of FARA grants by strategic area for the last six years.) FARA is not just a research funder but an active partner in the process. The FARA scientific staff regularly visits funded labs to learn their latest developments, challenges in the work and how to take strong work forward.

The collaborative clinical research network (CCRN), FARA's next largest program investment, resides in the middle of the continuum. The CCRN is an international network of 10 clinical research centers that work together to advance treatments and clinical care for individuals with FA. The network collaborates with pharmaceutical companies, government agencies and other research centers and the patient community to facilitate clinical research and trials needed to identify new therapies. The network and the patient registry are part of the infrastructure for clinical trials that in 2016 will have facilitated recruitment, enrollment and study participation in five clinical trials, >12 clinical research studies (biomarker studies) and >900 natural history visits.



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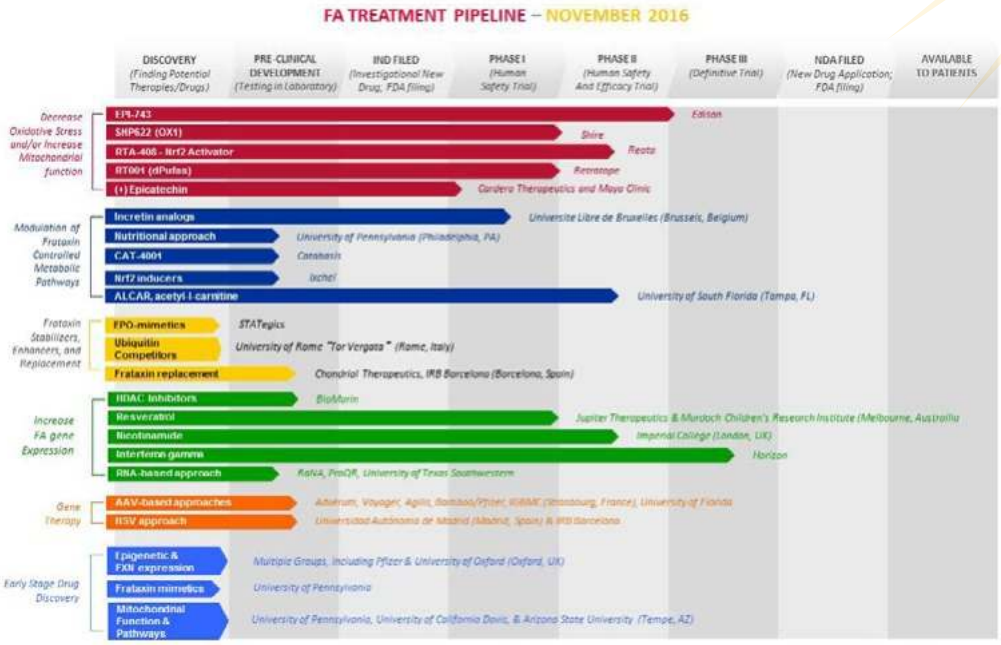


One of FARA's deeply held values is collaboration. This value guides many of FARA's programs and initiatives and two significant examples include FARA's work with pharmaceutical partners and FARA's collaborative efforts with international advocacy organizations. The treatment pipeline lists an average of 15 pharmaceutical partners with compounds in various stages of development. FARA regularly communicates with each of these partners and is often asked to participate in their FDA meetings, offer input on meaningful clinical endpoints, recommend clinic sites for trials, and recruit for those trials. Because 10-15 years ago research had not yet advanced to this stage, this program focus is more recent. An updated treatment pipeline is shown above and detailed notes on each program can be found at **curefa.org/pipeline**.

While FARA's grant funding has always been international, FARA's Board of Directors have placed increasing emphasis on other aspects of international collaboration. For example, FARA is again partnering with the Italian advocacy organization, Go-FAR, and Ataxia UK to host a three-day International Ataxia Research Conference in Pisa, Italy. FARA will also reformat the patient registry to be a Global Patient Registry. With multiple ongoing international studies and trials, patient engagement and participation is now more important than ever. FA isn't a challenge for just one family or one country. The effort is global. The more we collaborate between organizations, the more the field stands to gain.

FARA maintains its founding programmatic focus; however, the advances in research and FARA's growing capacity have expanded the scope of each program. While we still have work to do together we have made tremendous progress in understanding the disease, growing the FA community (all stakeholders), having a diverse and robust treatment pipeline, and creating the infrastructure to conduct clinical trials. ●

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/patient-registry

Collaborative Clinical Research Network
curefa.org/network

Clinical Trial Participation
curefa.org/trial

The FARA Research Grant Program

By Bronya Keats, Ph.D.

During 2016, FARA received letters of intent (LOIs) from 38 researchers and invited 25 of them to submit grant applications. Following the peer-review process, eight of these new projects have been awarded funding and we anticipate that up to seven more will be approved for funding before the end of the year. Additionally, continuation funds for a second year were provided for 12 projects. So far, FARA has provided a total amount of $4.96 million in research funding in 2016. This amount includes ongoing support for the Collaborative Clinical Research Network (CCRN) and the Penn Medicine/CHOP Center of Excellence, as well as the major Biomarker development initiative, spearheaded by FARA.

Grants funded by FARA are categorized into eight strategic research areas (including the CCRN) and funds allocated to each of these areas over the past six years are shown in the graph below.

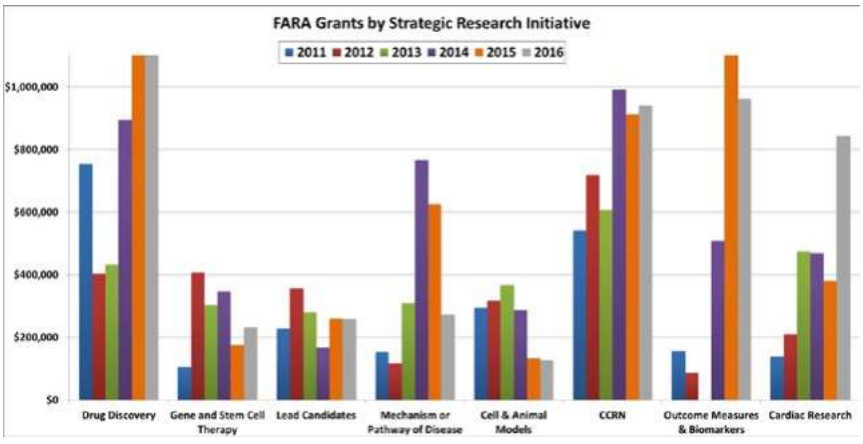
The 20 research projects that received funding in 2016 fall into the following categories: Drug Discovery 5, Gene and Stem Cell Therapy 2, Lead Candidates 2, Mechanism or Pathway of Disease 2, Cell and Animal Models 1, Outcome Measures and Biomarkers 3, Cardiac Research 5. Lists of all grants awarded by FARA each year since 2005 can be accessed at **www.curefa.org/grant** and summaries of most of these projects are available at **www.curefa.net/RPMP/public/pggrantlist.aspx**

FARA continues to partner with other advocacy groups around the world to fund outstanding research. Most recently, the 2016 award to Dr. Ben Deverman at the California Institute of Technology was co-funded with fara australasia and FARA NZ. Dr. Deverman's research is focused on developing novel viral vectors for delivery of frataxin to the nervous system and other sites affected in Friedreich's ataxia including cardiac muscle and the pancreas. Recently, Voyager Therapeutics entered into a licensing agreement with Dr. Deverman to advance his vector technology. We extend our heartfelt thanks to all the investigators who review grant applications submitted to FARA. Their voluntary contribution of time and effort is essential for ensuring the high quality of research projects funded by FARA.

Grant application guidelines and the deadline for LOIs and full applications for the FARA general and named research grants can be found at **www.curefa.org/grant.**

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.

Research publications resulting from FARA funding, as well as other publications on Friedreich's ataxia, are summarized at **www.curefa.org/scientific-news**



rideATAXIA— The Power of Team

By Jamie Young

"There is a power inside all of us that no scientist can explain."
—Antonio Maranhao Calmon, Living with FA in Brazil

Antonia Maranhao Calmon described the powerful energy felt by the community during the patient panel at the annual FA Center of Excellence- Children's Hospital of Philadelphia FA Patient Symposium, following a record-breaking rideATAXIA event in Philadelphia this fall. On October 16th rideATAXIA welcomed more than 615 cyclists to Blue Bell, PA. The cyclists raised over $320,000 for FA research, a total that left staff, FAmilies, volunteers and participants in awe since this was triple the amount FARA raised in its first year! After the cyclists returned from their 4-, 8-, 25- and 50-mile rides, the community celebrated with a delicious post-ride lunch provided by Outback Steakhouse, Carrabba's Italian Grill and Bonefish Grill.

FAmily teams are the key factor in surpassing event goals and they show a little friendly competition to be in the top spots. This year, 95% of the total fundraising in Philly came from teams. Our teams use their personal stories of living with FA to engage and motivate their local communities to be a part of research progress through donating, volunteering and joining their team. We celebrate the top five fundraising teams and all of the amazing 40 teams who collectively raised over $320,000. Thank you for your support, dedication and healthy competition!

rideATAXIA Philly's Top 5 Fundraising Teams

Cyclists start their 25-mile ride at rideATAXIA Philly.

Team Sean Anderson may not have pledged to wear chicken suits this year, but they were still shaking their tail feathers at the post-ride party as our number one team, raising over $65,000!

Team Laura, inspired by Laura Beth Jacquin, is not new to a top spot. They raised over $34,000 this year, continuing their friendly rivalry with Team Sean Anderson. This year they reached a significant milestone of $250,000 in total fundraising for FARA. They also bring one of the largest teams with over 35 participants.

Team Hope for toMORROW, led by Ben and Kristin Morrow, returns for the second consecutive year to the top five. The team's rideATAXIA fundraising reached over $27,000 in 2016 and over $57,000 total. This is in addition to hosting their own fundraiser in the spring. Team Hope for toMORROW raises funds in honor of Anna Morrow.

Team Ritschel is inspired by Samantha and Angelina Ritschel and captained by their father Pat Ritschel. Pat has not only been a powerful rideATAXIA fundraiser (over $20,000 in 2016), but continues to dedicate his time and expertise as an active member of the FARA Board of Directors.

The fifth team in fundraising, but number one in our hearts, is ***Team Riding on Fumes,*** led by Dr. David Lynch, Principal Investigator of the CCRN and FA Center of Excellence at Children's Hospital of Philadelphia. Dr Lynch and his daughter, Abby, recruit a team of clinic co-workers, researchers and FAmily participants every year at rideATAXIA Philly. This year the team raised over $18,000.

Continued on next page

Thank you to all of our rideATAXIA Philly Teams!

Sean Anderson
Team Laura
hope for toMORROW
Team Ritschel
Riding on Fumes
Defying Gravity
Sam's Squad
Team Barendt
Love for Logan
Team Liam '16
Race for Abby and Chase
RJ's Riders
Team Sweeny
Wheels of Fortune
Linda's Lifesavers
Spark Hope
Get Back Up
Team Timbie
Team Tiffany
Team Christina
Pfizer Pflyers
Gannon's Gang
West Chester Cycling Club (WCCC) / Johnson
Matthey
Team MacDonald
Team Hill
Cruisin Crusaders
Hampton Roads Hope
Team Terri
Dreams Come True
Team Early
Team Head
Merck Wheels of Steel
The Good Neigh Bears
Team Valley Vet
Team Monser
Breaking Away
Freewillers
PFA
Team Horizon
Team Voyager
Team Reata

2017 Upcoming rideATAXIA Events

Dallas	April 1
Atlanta	Spring TBD
NorCal	Spring/Summer TBD
Chicago	July 23
Philadelphia	October 15
Orlando	November 12

Get all the details at www.rideataxia.org.

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 $130,000* in memory of the following individuals:

Aaron Kittel, Alice Krim, Amee B, Andrew Serpa, Anita McHugh, Ann Marandola, Aubrey Olson, Barbara and Brenda Craig, Becca Van Schoick, Bert Wiley, Brandon Barger, Brett Reed, Carissa Aikman, Chelsea Lane, Claude Burke, Darlene Buss, David Seymour, Dell Redding, Dorothy Heberer, Douglas Spooner, Edward A. Thomas, Eileen Parsons, Eliza J. Foster, Everette McMahan, Fayrene Sorensen, Fred Consemi, Garret Timbie, Gregory Leonard, Heidi Merrill, Helen Fox, Holbrook Kohrt, Irene Bogucki, James Longmire, James Earl Walker, Joan Nelson, John Elliott, John Gates, John Houston, John "Jack" O'Brien, John Potter, Joseph R Williams, Joseph, John & Michael Forte, Joshua G. Gardner, Judith A. Russell, June Riermaier, Justin West, Karen James, Karsen Trentham, Keith Andrus, Kelly Sugrue, Kenny Treece, Kevin Maher, Kimberly Pokorny, Laura Busenlehner, Leah Chalcraft, Lois Orchard, Loren Magoon, Margaret Bence, Mario Petrini, Melanie Rose, Melissa Fox, Michael Despenas, Michael, Lynne & Mark Prather, Mildred Wharton, Milton Strebel, Mitchell Short, Nancy Terry, Nick Olson, Nicole Kent, Patricia Ann Kinsella, Patricia Jamison, Patricia Phillips Rust, Patty Casey Thompson, Peggy Johnson, Percy Penn, Phillip Bennett, Phyllis F. Burgoyne, Pop-Pop Andresen, Ray Nolasco, Rebecca Barbush, Robert Deemer, Robert Ponte, Robert Power, Ronald Kramer, Sandra Byrne, Sara Ferrarone, Scott Keehn, Ted & Virginia Prather, Thomas Barnett, Thomas Mueller, Thomas Piotrowski, Wayne Wang, William Gambill, William Pomeroy, William Wagner, Zac Pavelchak.

To request envelopes to be used for memorials or for more information about how to include FARA in your estate planning, please contact FARA at **info@curefa.org**.

*Year to date as of October 28, 2016

Van Schoick Family Fund Supports Research Travel

FARA accepted 12 submissions from FA Young Investigators for poster presentations at the annual FA Center of Excellence- Children's Hospital of Philadelphia FA Patient Symposium. Travel for these investigators was made possible by the Van Schoick Family Fund. The fund also provides travel support for FARA Ambassador training as well as travel stipends for patient participation in clinical studies. The fund was established in loving memory of Becca Van Schoick- daughter, sister, aunt, and Notre Dame alum. Thank you to the Van Schoick Family and their supporters for connecting the scientific and patient communities in such a meaningful way.

Lessons Learned from Advances in the Field

By Jane Larkindale, PhD

Drug development for neuromuscular disorders has been in the news a lot recently, with several drugs beginning to show great promise. This is great news for Friedreich's ataxia, in addition to being wonderful news for patients and families with other diseases. Although each disease is different, success in any one field has immediate ramifications for other disease areas – there is a lot we can learn from each other.

Spinal Muscular Atrophy (SMA) is a genetic disease caused by a reduction in a protein called SMN (survival of motor neurons). Two different ways of increasing SMN production have been in the news, reporting good results from recent clinical trials. Both treatments use technologies that are also in development for FA (oligonucleotides and gene therapy), although the actual molecules used are disease-specific.

The more advanced of the SMA therapies is called nusinersen, and is an oligonucleotide therapy being developed by Ionis Therapeutics and Biogen. A clinical trial of nusinersen in infantile-onset SMA (patients that typically develop the most severe form of the disease and die by age 2) showed that over the course of one year many of the babies were passing motor milestones that most infantile-onset SMA patients never achieve (e.g. kick, roll over or sit up). In FA, just like in SMA, our goal is to boost the amount of a specific protein. Several groups are looking at specialized oligonucleotides to increase frataxin, but these therapies have yet to reach the clinic. When the FA groups design oligonucleotides that will increase frataxin enough, we will be able to move more quickly through development based on some of the following lessons learned with nusinersen and other earlier oligonucleotide therapies:

- ***Understanding of how to target oligonucleotides to have the desired effect has increased dramatically, and we have learned a lot about how to build such molecules. This means that we can test more molecules that are more likely to have the desired effect, more quickly.***
- ***Many early oligonucleotides proved to have nasty side effects. Nusinersen and other recent oligonucleotide therapies have a slightly different chemistry, and the newer drugs seem safer.***
- ***In early SMA trials there was some concern about how to deliver the oligonucleotides to the central nervous system. They have designed and tested pumps that can safely administer the oligonucleotides at a safe dose directly to the brain, a technique which may also apply in FA.***

A small company called AveXis has also reported very positive results in an SMA trial. This company is developing a gene therapy, which in a Phase I trial showed infants passing early motor milestones. We have several companies looking to develop gene therapies for FA using similar technologies. AveXis' and gene therapy results for other diseases will help FA gene therapies move forward by:

- ***Demonstrating that a gene can reach therapeutic levels in the central nervous system after AAV gene therapy at a dose tolerated by patients.***
- ***Exploring different ways that AAV gene therapy can be administered.***
- ***Showing how the immune system reacts to such treatment.***
- ***Informing us as to safety concerns that may need to be monitored in early trials.***

Almost every week there is a report of new results from some study of relevance to our community, which will inform development of our ever-deepening pipeline of potential drugs for FA. The more we know about a technology and how to deliver such a drug before we even start testing in FA patients, the faster and smoother development of potential therapies for FA can be expected. We are living in exciting times!

Join The FARA Patient Registry!

Get notified about new trials and help advance FA research!

www.curefa.net/registry

The 2016 FARA Energy Ball welcomed a sold-out crowd that raised $2 millon in support of FARA's mission to treat and cure FA. The series of events kicked off with the USF Educational Symposium, followed by a Scientific Biomarker Meeting, a private screening of The Ataxian at Amalie Arena, a Tampa Bay Lightning hosted Patron Party for sponsors, followed by the Energy Ball itself. Thank you to Event Coordinator Ava Forney, Emcee Wendy Ryan and the Energy Ball Committee for bringing this year's Cocktail Cure themed event to life.

Excerpts from Ambassador Leadership Team member Alex Fielding's blog capture some of the magic of Energy Ball weekend.

The FARA Energy Ball: The Cocktail Cure

By Alex Fielding

I dressed in my finest suit, and even managed to iron my shirt and tie without burning myself. Good thing, because the crowd at the Energy Ball was dressed to impress! Everyone arrived in downtown Tampa and started mingling during the cocktail hour and silent auction. And when I say crowd, I mean CROWD – researchers, FA individuals, families, donors, friends, neighbors, business partners, FARA supporters… there must have been 50,000 people there! (Perhaps a slight exaggeration – closer to 750 people.) Patrons started the donations by bidding for items in the silent auction using a live app on their phones. There were so many auction items, the tables holding them stretched further than Tom Brady could throw a football! (Yes, I am a Patriots fan, and that is pretty far.)

After meeting fantastic new faces, sharing laughs (and selfies), we took our seats for a delicious steak dinner. While we were eating, our emcee and auctioneer were hard at work raising money for FARA to support research and drug development. Live auction items included getaways to tropical paradises, remote mountain escapes, premier golf courses, and major league sporting events! The most impressive part was the Fund A Cure live donation drive. For nearly 30 minutes, generous donations were made directly to FARA and the live total was continually displayed on screen. At the first bite of salad, the screen read $48,365…at the

Event Emcee Wendy Ryan with Energy Ball Founders Suzanne and Paul Avery.

end of the salad, $132,479… on to my steak, at $248,847… potatoes pair nicely, bringing us to $384,592…finish my steak with $486,073…by the end, over $550,000 raised! In the end, through sponsorship, dinner tickets, silent auction, live auction, and direct donations, over $2 million was raised for FARA! This money funds research labs to find new discoveries, supports therapy development to produce and test new potential drugs, and funds the travel and testing costs for clinical research patients participating in trials that validate potential drugs to an approved treatment.

The night culminated in a well-deserved celebration with dancing and live music by Blonde Ambition. The FAmily flooded the dance floor and even joined the singers on stage, feeding off the energy from the night. As I staggered back to my hotel room (from FA, not from the beer…) one conversation stood out in mind:

Patron: "My family and I saw you speak the other night at the symposium. Thank you so much for having the courage to share your story with us. We are always inspired when we hear from the patients."

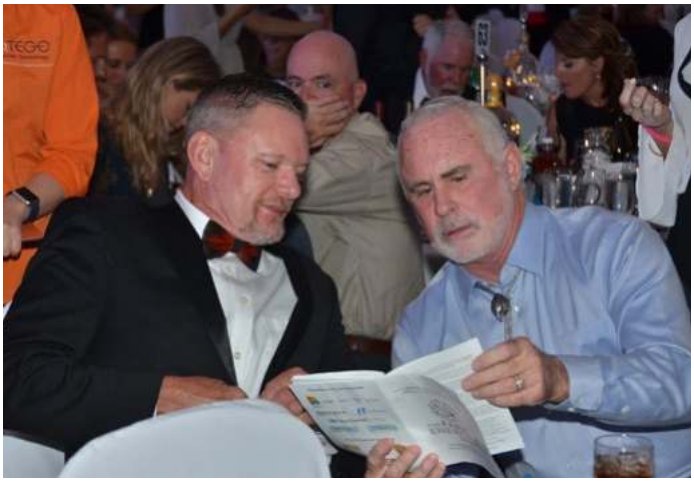
Me: "Thank you. I'm honored to be invited and given the opportunity to add to this community. How are you connected with FARA? Does someone in your family have FA?"

Patron: "No. There is no one close to us with FA."

Me: "Are you working with one of the researchers? Associated with a lab or pharmaceutical company?"

Patron: "No. We are not involved with any of the ongoing research."

Me: "Then may I ask what brings you here each year?"



FARA supporters Tom Bradley and Tom Pepin review the live auction catalogue.

Patron: "We truly believe in this cause. We wanted to get involved with a challenge and be part of the solution. When we first came in contact with FARA, we felt connected to the community. Experiencing the strength from patients inspires us. Hearing the dedication and progress from the researchers, gives us hope. We go to the symposium because we want to know what progress has been made to get us towards a cure."

I realized a key distinction from this dialogue: FARA does not have supporters, they have investors. They are not just supporting FARA's mission, they are invested in it. They are not just supporting work towards a cure, they are invested in finding it. This is the Together in *Together we will cure FA!*

The FARA Advocate is brought to you by:
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Cover Design: Crystal Wade
Design/ Layout: Anne Myers
Graphic Support: Lawrence Phillips
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Featured Scientist—Dr. Matt Hirschey

By Eileen O'Connor

Matt Hirschey's favorite grade school class involved what he describes as "crazy logic puzzles" in which you try to problem solve and restore the order of things. For as long as he can remember he has been drawn to the challenge of problem solving – and today it is this passion to answer some of the most challenging questions in the field of Molecular Physiology that motivates Hirschey in his work at Duke University Medical Center, where he is leading a team in FA research.

Dr. Hirschey, an Associate Professor in the Departments of Medicine and Pharmacology, traces his interest in physiology and drive to understand how the body works to his background as a competitive athlete. A lifelong runner and track and field competitor at the University of Vermont, he has always been fascinated by how the body metabolizes and produces energy. Originally Hirschey intended to pursue a medical degree, but his passion for research and problem solving led him to earn a PhD in chemistry, which he received from the University of California in Santa Barbara.

As an athlete and father of two young children, Hirschey has an intimate understanding and appreciation for how a body functions in optimal health – and what it can and must be like to feel and to see a healthy body begin to fail. Over the past few years Hirschey has had the opportunity to meet patients and families living with FA and describes how these interactions help to keep him focused. "As a father it's tough to meet and to see these parents – to know how hard it must be. Hard doesn't even come close," he quickly added.

Last year Hirschey and some of his colleagues attended the FA international meeting in Windsor, England and this past summer met FARA staff and FA ambassadors at an event in North Carolina. "I think it (these interactions) matters. We are in the lab tinkering away with models and biochemical reactions and it's hard

FA Parent Matt Price, Dr. Matt Hirschey, Jen Farmer, FARA Ambassador Mary Bircher, Angel Martin- PhD Candidate, Paul Grimsrud, PhD, and FARA Board Director, Pat Ritschel.

because science is slow. We try to be rigorous and meeting actual patients helps us to stay motivated."

In his five years at Duke, Hirschey has become a leading researcher on the process of acetylation of proteins in mitochondria, which fortuitously for the FA community, led to a partnership with cardiologist and FA researcher, Dr. Mark Payne. Four years ago Hirschey came across a paper detailing Payne's research and findings about an FA mouse model that was accumulating these tags or markers on cardiac proteins at a higher rate than normal. This did not make sense to Hirschey and his colleagues.

"With FA we found an interesting problem – which was screaming 'we don't understand how this works!'" said Hirschey. As the FA 'puzzle' presented itself, Hirschey, immediately was hooked. Hirschey and Payne wrote a grant to FARA to study the process of acetylation in mitochondria as it relates to heart disease. Their work has shown promising results. Given this writer's very limited scientific sensibilities, Hirschey tried to explain their findings in as simple a language possible:

"The markers are controlled by a protein called Sirtuin. There are seven different Sirtuin proteins in different parts of the cell that perform the same type of enzymatic activity. In FA patients at least one of the Sirtuins is inactive. The way to activate Sirtuin is to give a boost. A boost that has been identified as effective in this way is called NAD. NAD (an acronym for nicotinamide adenine dinucleotide) is like a vitamin. When you give the vitamin,

Continued on next page

you boost NAD and can shift metabolism and make disease states better. When FA mouse models are given a NAD precursor, early data show the amelioration of cardiac conditions, and it appears to offset cardiac problems that the mouse develops."

Simply put: "There is emerging data showing the positive effects of NAD therapy in FA."

When asked what he is most excited by in his current FA research, Hirschey immediately identifies these findings: "That the pre-clinical trials on NAD supplements show that a decline in heart function slows – and the fact that these findings could impact several other diseases – diabetes, obesity, heart failure and the effects of aging. Hirschey also notes that a paper published recently in Cell Metabolism that shows another ataxia (Ataxia Telangiectasia, A-T) significantly improved with NAD supplement therapy **(https://www.ncbi.nlm.nih.gov/pubmed/27732836)**.

While the intricacies of scientific research may not always resonate with everyone, this writer is convinced that with the imagination and passion of a scientist like Hirschey, tremendous strides and breakthroughs are not only possible – but very probable indeed.

Partner of the Year Award

ROPES & GRAY

We are proud to recognize Ropes and Gray as FARA's 2016 Partner of the Year. There are a number of unsung heroes in our FA Community, and we are grateful for the opportunity to celebrate the service of one of them. Ropes and Gray is a global law firm with over 1,200 attorneys on staff. The firm has provided pro-bono legal counsel to FARA, specifically creating a template for grant contracts. Each research grant awarded by FARA is accompanied by a grant contract between FARA and the receiving research institution. This helps to ensure all parties have a common understanding of the grant terms (i.e. the timing of fund disbursement, the scope of work to be completed, and the forward path for any discovery). Attorneys at Ropes and Gray, especially Shrevanni "Vani" Suvarna, have been assisting FARA since 2008. Last year Vani and her colleagues donated over 80 hours of time to developing and helping negotiate contracts for our biomarker consortium. These great people have provided their legal expertise but have done so with a respect for FARA's collaborative culture and an eye to the best interest of FA research advancement.

Criteria for Partner of the Year Selection

- ***Leadership and/or employees/constituents within the partner organization are directly supporting FARA's mission and demonstrate a commitment to curing FA faster than thought possible (e.g., fundraising, in-kind contributions, professional services, etc.).***
- ***Organizational culture that values community, helping others, volunteerism.***
- ***Commitment to increasing awareness of Friedreich's ataxia or FARA.***

The Annual Race for Matt & Grace Sets $100K Goal

By Ann Musheno

Hundreds gathered to run, walk, or roll in the 7th Annual Race for Matt & Grace in Providence, RI, on September 24. The event featured a digitally timed 5K and an after-party complete with raffles, live entertainment and food.

The event was launched by Katie Hopkins, cousin to Grace Hopkins, who was diagnosed with FA in 2008 at the age of 12. Participating in another 5K road race just days after learning of Grace's diagnosis, Katie could not help but ask herself "Could I do this?" After doing her research and learning all she could about FARA and grassroots fundraising, Katie pulled off the first "Race for Grace" with the help of family and friends.

At this first race, Katie met the DiIorio family. About 13 years prior, Matt DiIorio had been diagnosed with FA. Matt's parents, Jack and Sallyann, and his close friend Mike Crawley would come to play a huge role in the continuity and growth of the event. Now Katie, the DiIorios, Mike, and the ambitious committee they lead have set one lofty goal for 2016: raising $100,000!

In order to reach this goal, the group supplemented the race with several smaller fundraising events throughout the year including a comedy show, restaurant nights, Zumba nights, and more. The committee keeps supporters from burning out by diversifying their fundraising initiatives. Mike says they are constantly on the lookout for ways to keeps things interesting for their support base while providing an opportunity to help.

The group has employed the use of several companies' ongoing charitable programs, a low-maintenance way of continuously fundraising. Most recently, they got involved with HALO Text, which allows supporters to sign up to receive text advertisements, with a portion of the advertising proceeds going to FARA. For FAmilies new to fundraising, Katie recommends that fundraisers realize their limits, knowing that the FA community will be grateful for anything you do, large or small. She also recommends that you use available resources and ask for help. "Planning big events is a large time commitment and you have to make sure you have the time and support you need," she says.

Top fundraising teams for Race for Matt & Grace: Alex's Avengers and Matt's Marauders.

Race for Matt & Grace succeeds in large part because organizers ask for help. Jack says that asking for small in-kind donations or discounts sometimes prompts companies to fully underwrite expenses such as food and beverages, or even become a corporate sponsor of the event. Also key to the event's growth is the use of FARA's peer-to-peer fundraising software, which enables everyone registered for Race for Matt & Grace to set their own fundraising goal and reach out to their own network about supporting FARA through email and social media.

The group is on track to meet their ambitious goal, as is FARA's "Mission 1 Million" goal to raise $1 million annually through grassroots events such as The Race for Matt & Grace! To learn more about grassroots events across the country or how you can host your own grassroots fundraiser, visit **curefa.org/grassroots**. Together we will cure FA!

FARA Ambassadors Jean Walsh, Erin O'Neil and Christina Logan give speeches to rev up the crowd before the race began.

FARA Honors 2016 Friends and Allies

By Felicia DeRosa

We have encountered many good citizens and neighbors in our effort to treat and cure FA. Some volunteer for fundraising events, others donate and still others raise awareness. Then there are our friends, our allies who make whole parts of our organization and events happen. They invest their time and their resources, but they also invest themselves.

We are incredibly fortunate to have these friends, these allies as part of the FARA Energy Ball. Someone doesn't stay up late to crunch auction data, or get up early to design posters, or load and drive a 26-foot moving truck, or sponsor the Ball and donate to the event's Fund a Cure without a love for the whole- the mission, the work, and the people. Kurt and Amy Hull and Frank and Anna Alfano have done all of these things, and we are honored to present then with this year's FARA Friend and Ally award.

The Hulls and Alfanos have been long time generous supporters of FARA through the Energy Ball and other Florida based events like rideATAXIA Orlando and Pull for a Cure. They have adopted FARA's mission in a very personal way—one where they've invested countless hours in service to and invited friends and family to support. They believe in us. They know we are making a difference together. And their actions speak volumes about their friendship.

Amy and Anna are entering their third year as the auction co-chairs for the Energy Ball, and it is an auction that has it all—exclusive getaways, autographed memorabilia, and unique dining experiences. One wouldn't necessarily think about an auction item's journey—how it was secured from a generous donor, how the item information got transformed into a beautiful poster inviting you to bid, and how it took its place on a table among 300 equally cared for items lining the length of an expansive ballroom hallway at the event. The secret is FARA's Friends and Allies. Amy and Anna are a dynamic team with complimentary talents. Amy leads a committee to secure the right variety of auction items, ensures that the data is accurate and items are priced appropriately for bidding. Anna brings her creative expertise to the display materials like poster boards as well as the winning certificates. This year their auction raised over half a million dollars!

2016 Friend and Ally Award recipients Frank and Anna Alfano, Amy and Kurt Hull with Paul and Suzanne Avery and Sam Bridgman.

We are sincerely grateful to Kurt, Amy, Frank and Anna for not only being a significant part of the Energy Ball's success, but also FARA's mission to treat and cure FA. They have shown that they are Friends, Allies and an integral part of our FAmily. •

FA Woodstock—Peace, Love & Research Blood Samples

By Kyle Bryant

There are two things that give me a positive outlook when facing FA:
1. Meeting others who live with FA.
2. Participating in research.
FA Woodstock is a prime opportunity for both.

I had the opportunity to go to FA Woodstock the very first year. In 2010, it consisted of a few people hanging out around the Hook's pool, shooting the breeze, enjoying each other's company and camping in tents. When I got home, I noticed a lot of buzz on Facebook. We all loved it so much that we wanted to tell others. The event has grown from 30 attendees the first year to 180 attendees in 2016. There are new additions to the FA family each year and new bonds are always being formed. There is something about looking someone in the eye and knowing they understand something about you that not many people understand; that never gets old for me.

FA Woodstock takes place at Flying H Ranch in Northwest Indiana, the home of the Hook family. Over three days in July, there are lots of organized activities all over the property at different times throughout the days staffed by hard working volunteers. These activities include swimming, fishing, tie dye, art projects, yoga, pizza, poker, fireworks, and all the soft serve ice cream you can handle. This, along with amazing food, is all provided for a more than reasonable registration fee. Participants now have the option to camp on the property or stay at a nearby hotel.

In addition to these activities, there is an opportunity to participate in research. FARA's Executive Director, Jen Farmer, Dr. Dave Lynch from Children's Hospital of Philadelphia(CHOP), and the research team from CHOP set up a mobile clinic to take blood samples, cheek swabs, skin punches, neuro exams, and hearing tests. It is a two way exchange as the whole team is also there to field questions.

When living life with a rare disease like FA, it is important to feel camaraderie and to know that your research contribution is getting us all closer to the finish line. The community is in debt to the Hook family and all their friends and volunteers for creating the opportunity for both.

TThe FAmily works on their best poker faces during the FA Woodstock poker tournament.

Attendance is getting so large that the annual photo needs to be taken from the rooftop.

Dr. Lynch, Jen Farmer and the CHOP team hard at work drawing blood samples for research.

FA Woodstock will take place Thursday, July 20–Saturday, July 22, 2017. For more information about FA Woodstock visit **hooksflyinghranch.com** or email Paula Hook at **ckjhook@aol.com**.

Outback Steakhouse's Southeast Region took part in an adventure race on Sept. 7, raising over $60,000 for FARA!

Chelsea Conley and Anna Gordon show off their Halloween finest at Costume for a Cure on Oct. 23. (St. Clairsville, OH)

Jason, Hannah, and Austin Stacks throw out the first pitch at the 7th Annual Swing Away at FA on Oct. 22, an event featuring a wiffle ball tournament and fun for the whole family. (Dawsonville, GA)

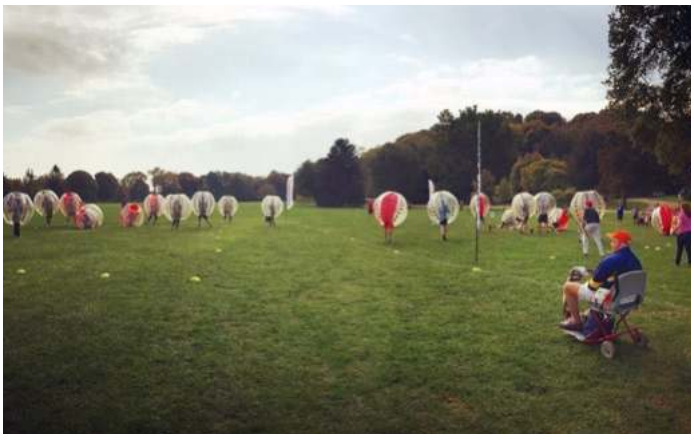
Several FAmilies joined together for the Slim's Journey FARA 5K Run/Walk on Sept. 24 hosted annually by the Myers Family. Photo Credit: ADI Photography (Warrenton, MO)

Jacob Tompkins and Dylan McDonnell at a concert on Aug. 18 benefitting Team Adirondack's 7th Annual Hike for Team FARA. The McDonnell Family also hosts an annual music festival in September which includes kids' games, face painting, and a silent auction in addition to awesome live music! (Queensbury, NY)

FAmily takes a break from zip-lining to get a selfie at FA Adventure Day on Aug. 22. (Beverly, MA)

Team Attackin' Ataxia finishes strong at rideATAXIA Chicago on July 23. (Channahon, IL)

Top fundraisers at rideATAXIA Chicago pose with rideATAXIA Founder & Director Kyle Bryant. This year's ride in Chicago raised $95,000! (Channahon, IL)



Rick Peters supervises a game of knockerball at "Knock out FA" on Oct. 16. (Peoria, IL)

Friends and FAmily gathered at the 10th Annual Welsh Bash in the Backyard on July 23 for live music, beer, raffles and cornhole. (Harrisburg, PA)

Kyle Bryant with the patient panel (Alex Fielding, Sean Baumstark, Alison Avery and Anna Gordon) at USF Health's "Understanding Energy for a Cure" Symposium on Sept. 15. This free event is held in conjunction with the annual FARA Energy Ball. Photo Credit: Eric Younghans (Tampa, FL)

Individuals and teams swam, ran and cycled in the Westchester Triathlon on Sept. 25 as part of Team Donovan and Team FARA. (Rye, NY)

On Oct. 15, the FARA Ambassadors came together for a training day on drug development & clinical trials. (King of Prussia, PA)

Two-legged and four-legged friends of Jason Drake came together on July 16 for a running of the foxhounds to benefit FARA. (Jarratt, VG)

The winning team at the 34th annual Claxton Classic Golf Tournament accept their award. (Mitchellville, MD)

Fine Arts for Friedreich's Ataxia held their first event, Curtain Call for a Cure, on June 17. The event featured 20 talented high school students staging several well-known musical theater numbers. (Sioux Falls, SD)

FAmily gathers at the 30th Annual Fuzzy Buzzy Golf Tournament on Sept. 11. This was the 13th year that the event supported FARA on behalf of Erin O'Neil. (Windham, NH)

Erich Pieper enjoys a sail with Y-Knot, an accessible sailing program on Lake George, on Sept. 17. His sail raised funds for Team FARA and Y-Knot. (Kattskill Bay, NY)

FAmily enjoying a day of clay shooting and golf at The Rocky Mountain Bird & Birdie on Sept. 23. (Commerce City, CO)

Jennifer Guy shows off her medal after completing the Ironman Weymouth 70.3! In just one day, she swam 1.2 miles in the sea, biked 56 miles and then ran a half marathon as part of Team FARA! (Dorset, UK)

Josh Lamascus and Julio Cardenas with some of the fabulous prizes at the Century 21 King Charity Golf Tournament on Sept. 22. (Fontana, CA)

The Rao Family welcomed over 300 attendees to their first 5k walk on Nov.5. (Sugar Land, TX)

Hundreds came out to support Alison Price at the Run for You 5K on Oct. 29. All proceeds from this event went to support Team Alison P's Team FARA fundraising. (Wake Forest, NC)

Doug Finck completed The Chicago Marathon on Oct. 9 as part of Team FARA. Here Doug smiles through mile 21.5! (Chicago, IL)

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WINTER 2016 - 2017

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

This Issue:

FARA Friedreich's Ataxia Research Alliance

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