WINTER 2017 - 2018

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

This Issue: Research Updates • Featured Scientist - Dr. Ben Deverman • FARA Energy Ball- Knock Out FA

FARA Friedreich's Ataxia Research Alliance

CUREFA.ORG

President's Message

By Ron Bartek

Dear friends,

Most of you know how long and hard FARA and the FA community have worked to establish and nurture strong, helpful relationships with our colleagues at the Food and Drug Administration (FDA) – the people who review all our proposals to initiate clinical trials and who will review all our applications for therapy approvals. We are excited to report that recent, extremely positive developments in those relationships could not be better timed and that many of you have played and will continue to play important roles in those developments.

The recent developments were built upon a strong foundation of mutual respect. FARA has long worked with and on behalf of the FDA, participating, for example, in numerous FDA meetings with our pharma partners in submitting clinical trial proposals, meeting with FDA colleagues as they were designing various programs for patient engagement, and advocating in congressional offices for increased resources for the FDA. The FDA has long seen FARA as a model patient organization, frequently asking FARA to present at FDA internal and external meetings to demonstrate how such organizations can work effectively in collaboration with the FDA. The FDA has even sought FARA input and advice when designing specific programs for the rare-disease community.

A real milestone in this extremely positive FARA-FDA relationship was achieved on June 2 when FARA assembled hundreds of FA patients and families in person and online along with about two dozen FDA officials and a number of FARA's pharma partners to participate in our Externally Led Patient-Focused Drug Development Meeting in College Park, MD. This event was one of the first in a series of such meetings organized and funded by individual disease organizations and intended to provide FDA personnel the opportunity to achieve a deeper understanding of those diseases by listening to the voices of the patients and caregivers. Our FA patients and caregivers did a spectacular job - both those who presented in person and those who participated online – as you can see in our "Voice of the Patient Report" (read at http://curefa.org/pdf/news/FA-Voice-of-the-Patient.pdf or watch at https://www.youtube.com/watch?v=Va1D4SqrSfw).

This meeting had a powerful, immediate effect and continues to benefit the FA community tremendously. In conversations, since, that included some of our pharma partners, FARA staff and key FDA leadership personnel who were with us for the entire June 2 meeting, our FDA colleagues made it clear that they had "heard the voice of FA patients loud and clear" and that the meeting "had a significant emotional and scientific impact on all of us that is changing the way we will do our work with FA." In those same conversations, those FDA colleagues were also able to provide our pharma partners with clarifications on FDA guidance that provide FARA and our partners with a much clearer path forward in developing treatments. Also, during an October 30th FDA Rare Diseases Public Workshop, FARA and its June 2nd meeting were singled out and commended from the podium by Dr. Billy Dunn, Director of the Neurology Products Review Division of the FDA's Center for Drug Evaluation, which reviews all proposals and applications regarding drug therapies for neurological targets.

We are extremely grateful to all the FDA personnel who attended the June 2 meeting and have provided such encouraging remarks since that day, including Dr. Wilson Bryan, Director of the Office of Tissues and Advanced Therapies in the Center for Biologics Evaluation and Research (the Office that reviews all proposals and applications regarding gene therapy), who gave the FDA's welcoming remarks, and Dr. Jonathan Goldsmith, Director of the Office of Rare Diseases Programs, who gave the FDA's closing remarks. FARA Ambassadors sent hand-written thank-you notes to all FDA participants and we have heard back from a number of them that that they really appreciated these notes. Of course, we are, as always, deeply appreciative of all of you in the FA community who participated in this fantastic event and who help in so many other ways to support our relentless drive to treat, cure and eliminate FA.

Warm regards,
Ron

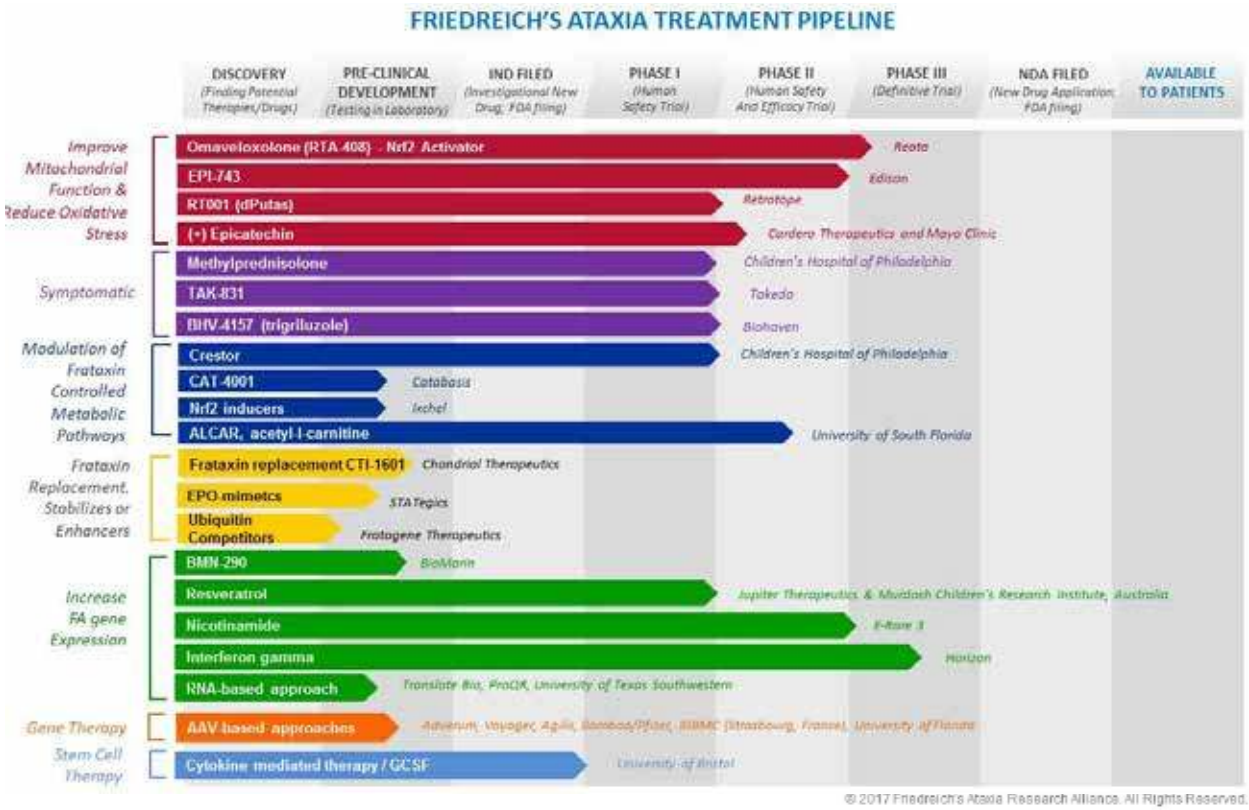
International Ataxia Research Conference and Advances in the FA Treatment Pipeline

By Jennifer Farmer

What a year! Your FARA team has been going nonstop, fueled by amazing community support and engagement and scientific progress. September and October were especially busy and exciting with scientific symposiums, investigator meetings for two clinical trials and great events like the FARA Energy Ball and rideATAXIA Philly.

The International Ataxia Research Conference (IARC2017) was held September 25-29th, in Pisa, Italy and co-organized by goFAR, Ataxia UK and FARA. The IARC2017 welcomed 400 attendees- researchers, clinicians, patients, and government, regulatory, and industry representatives from 22 countries. There were >30 platform presentations and over 95 poster presentations on FA.

In addition to the impressive engagement numbers, there were more than 20 platform and poster presentations demonstrating progress and informing our growing and advancing the FA treatment pipeline. You will see here an updated FA treatment pipeline. This pipeline is also on the website, curefa.org/pipeline, with updates from the past year for each of the candidates.



A few key updates include:

Omavaloxone – (aka Omav or RTA-408) is a therapeutic candidate being advanced by Reata Pharmaceuticals. Omav is a drug that improves mitochondrial function by activating Nrf2, a pathway that is downregulated in FA. Results of the part 1/Phase 2 study were reported in June and based on those encouraging results, Reata is advancing a Phase 3 trial with FDA supported endpoints. 100 individuals with FA will be enrolled, ages 16-40. FARA and Reata held a webinar to provide the FA community with detailed information about the program – the recording, list of active trial sites and eligibility criteria can found at: curefa.org/network.

TAK-831 – TAK-831 is a new investigational medicine being advanced by Takeda. TAK-831 may increase D-serine, an amino acid that activates specific receptors in the brain that may be important to the functioning

Continued on next page

and signaling of neurons. A Phase 2 clinical trial of TAK-831 studying efficacy, safety and tolerability is opening at the end of 2017 at multiple US sites and enrolling individuals with FA ages 18-55.

BMN-290 – BMN-290 is a selective chromatin modulation therapy that works by inhibiting the silencing of the frataxin gene. In cellular models, BMN 290 increases frataxin expression more than two-fold and the compound has been shown to penetrate the central nervous system and cardiac tissues.

This is the latest generation of HDAC inhibitors that were originally discovered by Dr. Joel Gottesfeld. At the IARC2017, BioMarin presented their work developing this series of compounds and in October they announced BMN-290 as a clinical candidate with plans to file an Investigational New Drug (IND) application for BMN 290 with the U.S. Food and Drug Administration in the second half of 2018.

GCSF - Granulocyte colony stimulating factor' (GCSF) is a new therapeutic candidate that has been added under stem cell approaches. This is based on research out of the University of Bristol. Experimental studies undertaken within Dr Wilkins' laboratory indicate that bone marrow stem cells protect nerve cells and induce repair of the nervous system. They have recently completed a major study of a mouse model of FA in which the bone marrow stem cell mobilizing drug, GCSF, protected mice from neurological damage. These are drugs that are used in clinical practice to activate stem cells within the bone marrow and induce them to circulate around the body. The Bristol researchers were recently awarded a new research grant from Ataxia UK and FARA to continue this research in human cell lines and a small pilot trial in FA patients.

The FA treatment pipeline is robust with many types of therapeutic approaches which are critical to our mission of treating and curing FA. We are encouraged by the number of drugs in clinical trials, as those may offer near-term treatments, and we are committed to the growing numbers of approaches employing genetic therapeutic strategies to increase frataxin.

Research Grant Program

FARA's Research Grant Program Expands

By Angel Martin, PhD

Following the completion of her FA research in Dr. Matt Hirschey's lab at Duke University this summer, Angel Martin, PhD joined the FARA staff as Research Director to help manage a growing grant program.

FARA granted $6 million in research funding in 2017 — a 25 percent increase from last year, an amount which reflects an increase in dollars donated and raised by the countless efforts of our FA and broader communities. Increased fundraising made it possible for FARA to award new grants across several areas of strategic importance (see graph below). We funded 15 new grants under the following strategic initiatives: Drug Discovery (2), Gene and Stem Cell Therapy (1), Lead Candidates (1), Mechanisms or Pathway of Disease (6), Cell and Animal Models (2), Natural History and Biorepository (2), and Outcome Measures and Biomarkers (1). Additionally, continuation funds were provided for 19 projects spanning nearly all of these strategic areas. These efforts will help FA research move forward by deepening our understanding of disease progression and by engaging innovative approaches to therapy.

Increased spending in these areas conveys promising progress for FA therapies, with the overarching goal of accelerating drug development from the scientist's bench towards the clinic. We are excited to be investing in promising new approaches to FA therapy. For example, under our Gene and Stem Cell Therapy initiative we awarded the 2017 Kyle Bryant Translational Research Award to gene-editing company CRISPR Therapeutics, an award co-sponsored by the Cure FA Foundation. In collaboration with Dr. Marek Napierala at the University of Alabama Birmingham, CRISPR Therapeutics will develop and evaluate pioneering gene-editing tools that target the genetic defect in FA in preclinical models. For more information regarding this exciting partnership, please visit: http://www.curefa.org/grant.

FARA continues to partner with other advocacy groups to fund outstanding research. These collaborative grant sponsorships have enabled investigators to successfully apply for competitive exter-

Continued on next page

nal funds to bolster research efforts. For example, a $2 million research grant awarded to Dave Lynch, MD at the Children's Hospital of Philadelphia (CHOP) from the US Food and Drug Administration (FDA) provides continued support for natural history studies in FA, with a focus on pre-symptomatic and early FA in children. These studies are conducted by Dr. Lynch in the FA Center of Excellence (CoE) at CHOP, as well as at several other sites in our Collaborative Clinical Research Network (CCRN) in FA. In addition to this award from the FDA, these studies at CHOP—along with biorepository studies—will continue to be jointly funded by FARA, The Cure FA Foundation, and the Hamilton and Finneran Family Foundations.

FARA is also excited to announce a grant awarded to clinical data scientist Christian Rummey for his research efforts to analyze the natural history data collected by the CCRN. His work so far has enlightened our understanding of the natural progression of disease in FA. For example, his analyses have enabled us to help pharmaceutical company partners design clinical trials that will yield more definitive outcomes. One important finding from his work underscores the importance of factoring in the natural progression of symptoms experienced by FA patients over time when designing clinical trials. When we do this, we can better recognize the length of time necessary to appreciate any meaningful improvement in disease outcome with therapeutic intervention.

Lastly, FARA has worked this past year to overhaul our grant program webpage with the goal of making this webpage more interactive and accessible to the broader FA community. Updates include responsive links for grants awarded in 2016 and 2017 calendar years, links that reveal a summary of the awarded grant, a photo of the investigator, and a brief list of the investigator's recent publications. You can view these upgrades, as well as download PDF lists of all grants awarded by FARA each year since 2005 on our grant program webpage (http://www.curefa.org/grant).

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

Clinical Studies are Critical to Research Advancement!

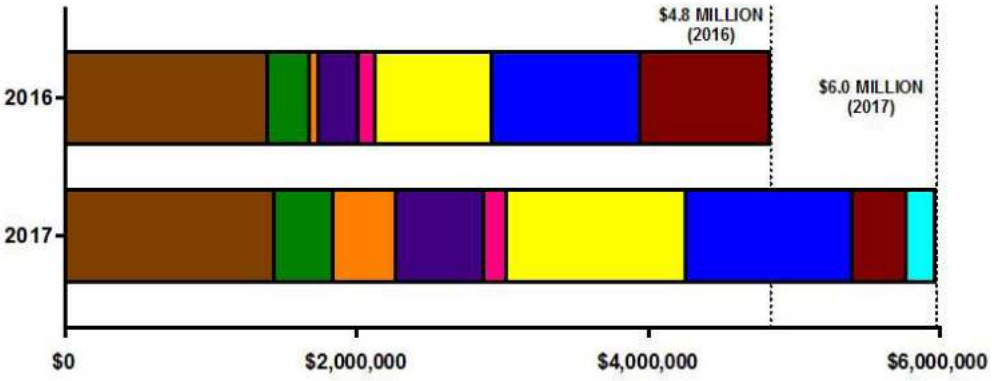
For more information on the latest clinical research and to learn how you can participate, please visit:

Patient Registry
curefa.org/patient-registry

Collaborative Clinical Research Network
curefa.org/network

Clinical Trial Participation
curefa.org/trial

FARA GRANTS BY STRATEGIC INITIATIVE



- Drug Discovery
- Gene & Stem Cell Therapy
- Lead Candidates
- Mechanism or Pathway of Disease
- Cell & Animal Models
- Natural Hx & Biorepository
- Cardiac Research
- Outcome Measures & Biomarkers
- Improving Clinical Outcomes

rideAtaxia – 10 Things I've Learned in the Past 10 Years

By Kyle Bryant

Kyle here. Crazy to think it's been 10 years since my mom, dad, and I trekked from San Diego to Memphis in support of FA research and awareness. RideATAXIA started with just one team—me and three other people— but now the program includes over a hundred families in six different locations who together welcome more than 2,500 riders each year! This year each person received a 10-year commemorative t-shirt to help celebrate our anniversary. I wanted to thank all of the people who have put their heart and soul into this program to produce amazing results. I also wanted to share the top 10 things I've learned in the past 10 years:

1. ***The FA community is a powerful force!*** In the beginning our team consisted of just my family. We had limited fundraising power. Now the entire FA community has joined me and my family and our reach is many times more when we act together. The proof is in the fundraising for 2016 and 2017: over $1,000,000 each year!

2. ***Outback Steakhouse, Bonefish Grill, and Carrabba's Italian Grill are dedicated teammates.*** We are told time and again that we have the best food at our events. It is no surprise because we have amazing partners who keep the riders coming back for more!

Team Tex 2 Cure FA getting ready to enjoy a beautiful SoCal Ride

Kyle leading the start in Philly

These three restaurants have been by our side from the beginning and continue to show amazing support at every ride.

3. ***Believe it or not, pickle juice is the refreshing tonic of choice.*** In Dallas they asked for and they received pickles at the rest stops so they can take shots to replenish electrolytes on a hot day. Yum!

4. ***Porta-Potties are important!*** Our rule of thumb is to have a fully stocked rest stop about every 12 miles, with supplies to refuel each rider and send them on their way. However, at the first single day ride, we did not even have porta-potties at the rest stops. What were we thinking! Now the accommodations are plentiful.

5. ***The power of volunteers.*** It takes about 100 volunteers to pull off a successful ride. We are incredibly grateful to the armies of people who come out to help on ride day at registration, rest stops, setting up tables and chairs, and cleaning up.

6. ***Determination and teamwork are a winning formula.*** Many people push themselves beyond their perceived personal limitations at the ride. Whether that means five miles or 50, we are inspired by each other. We each set our own goals but we also rely on each other to reinforce the motivation between teammates.

7. ***Sponsors make the difference!*** The ride program would not be possible without the generosity of our loyal sponsors. Sponsorships help us cover costs so we can dedicate your hard earned fundraising to research. We are especially grateful to the sponsors who join us on ride day with a team or a booth at the event.

Continued on next page

8. ***The power of one.*** Over and over we see one person mobilize his/her home community to participate and raise funds at the ride. We have individuals who bring a friend and raise $50 and teams of 30 who raise over $30,000. Every effort counts, so find a ride near you- in many cases it may be 3 or 4 hours away, but your presence is needed and welcomed! We hope to see you there.

9. ***It's all about details.*** This program would not operate without the tireless effort of rideATAXIA Program Coordinator Jamie Young—filling out permits, organizing volunteers, food, drinks, and rest stops; the list is endless and Jamie keeps it all on track.

10. ***It's an incredible journey.*** Even though my reason for starting rideATAXIA is serious—I want to cure an incurable disease I have— it's led me through some of the greatest personal experiences of my life and allowed me to meet some of my greatest friends. The work we do is so important, but rideATAXIA has also become one of the highlights of my life.

We can't wait for the day when we no longer need rideATAXIA but until then we are confident that this community will continue turning the crank.

Top fundraising Team Sean Anderson celebrating a great Philly ride with over $108,000 in fundraising

Members of Team Bonacorda's Army enjoying their first rideATAXIA. They raised over $35,000!

2018 Upcoming rideATAXIA Events

SoCal	February 24
Dallas	TBD
NorCal	June 2
Chicago	July 22
Philadelphia	October 14
Orlando	November 11

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 $170,000* in memory of the following individuals:

Aaron Kittel, Adelaide Di Sano, Alex Culbreth, Alice Krim, Andrew Serpa, Anthony Cidonio, Arlene Huber, Arlene Schofield, Aubrey & Nick Olson, Becca Van Schoick, Betty Lou Schlatterer, Billie Sue Alexander, Bobbie Smothers, Brenda Harrison, Bridget Brophy, Carol DeFilippo, Cecelia Divincenzo, Charles Bryan, Charles Kuehn, Chelsea Lane, Clint Ziegler, Corey Gilardi, Dan Kirkland Wells, Dave Haynes, Donald B. Harris, Dorothea T. Buckley, Douglas Spooner, Eleanor McNamara, Elmer T. Gould, Eric Bogart, Frances McCafferty-Pellegrino, Garrett Timbie, Georges Maugee, Holly LeBlanc, Ida Wood, Jacob Byrne, James Longmire, James S. Reeves, Jason L. Meaders, Jeannette Shephard, Jeff Rosenkranz, Jennifer Alexander, Jerry Paskvan, Joan (Honey) Long, John Andresen, John Elliott, John Randazza, Joseph Crisafulli, Joshua Chalcraft, Joyce Olson, Judy Loftin, Katie Miller, Keith Andrus, Kevin Maher, Kevin O'Connor, Leah Chalcraft, Leo Dagley, Linda Rasone, Loran Martin, Loretta Loudermilk, Lorraine Mashuga, Marcia Young, Marian Stokes, Marion McMahan, Mary L. Murphy, Mary Norman, Matt Rupel, Melissa Fox, Mella Hartenstein, Michael Bush, Michael O'Neil, Milly Witkowski, Monica Jefcoat, Paige Myers, Patricia Sheley, Peggy Johnson, Phil Wauben, Phillip Bennett, Rebecca Fulcher, Regina Krivinko, Rick Sharp, Robert Petrini, Robert Whitney, Rodney Dewar, Roland Thurow, Sara Ferrarone, Shannon D. McDuffie, Sharnelle Fitzpatrick, Terry McDonnell, Thomas Barnett, Thomas Yiannis Hood, Tichelle Florence, William Overfield, William Wagner.

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**.

*October 29, 2016-October 23, 2017

Genetic Therapies and Friedreich's Ataxia

By Jane Larkindale, PhD

In the past year, gene therapies have been frequently covered in the news. On August 30, 2017, the first gene therapy was approved by the U.S. Food and Drug Administration (FDA), and others are very close. Several companies are working on genetic therapies for FA. The goal of such therapies is to make more frataxin protein in patient cells. Genetic therapies include gene replacement, where a healthy version of the gene is inserted and expresses the protein, and gene editing, where tools are inserted to correct the patient's own gene and make it produce more frataxin.

Gene replacement is the most advanced gene therapy in FA. This involves injection of a healthy copy of the frataxin gene into a patient, typically using an inactivated virus to get the gene into the right place in the cell and make it express the protein. Most gene therapies use adeno-associated viruses (AAVs), although other delivery systems also exist. Different versions of AAV target different body tissues to different degrees, and the body's immune system may react differently to different AAVs. This means that each company or group studying FA gene therapy has its own versions, which may produce different amounts of protein in different places. In FA, a challenge of this technique is that we need frataxin throughout the body, most importantly in parts of the central nervous system and the heart. Choosing an AAV and a way to deliver it [e.g. intravenous injection vs. injection into the brain or spine] that gets enough virus to all the affected tissues has been a challenge. However, new AAV's are being developed continuously, and significant progress has been made toward getting genes to the tissues that we care about.

Meanwhile, AAV gene therapy has been tested in other diseases with good results, and we have learned a lot from these successes, including information about how to deliver the virus to different tissues, how much is safe to deliver (based on the delivery system, not the gene itself), how the immune system responds to these viruses, etc. FA gene therapies are expected to be ready for the first stages of human testing soon. FA is considered a good candidate disease for these approaches because all FA patients already express some frataxin, which means immune reactions to the protein are unlikely, and production of more frataxin even in a proportion of cells in an affected organ might be expected to help patients.

Gene editing is a newer technique, which is in the early stages of being studied in FA. Instead of inserting a replacement gene, gene editing aims to remove the mutation from the patient's own cells—in most cases of FA, the GAA repeat sequence in the frataxin gene. Gene editing tools are adapted from naturally occurring enzymes in bacteria, and allow very specific gene sequences to be edited. The therapy consists of a targeting sequence, which tells the tools what to edit, and "molecular scissors" that cut out or replace the indicated section of the gene. These changes would be permanent. The technique has been very successful in the laboratory, where individual cells have been edited effectively using several tool kits (CRISPR-cas9, TALENs and zinc finger proteins). In some diseases, gene editing has advanced into mouse models of disease, where some gene correction can be detected. However, challenges remain with getting the tools to where they are needed to edit enough cells to make a difference in clinical disease. Insertion of the tools is typically done using AAV vectors so delivery issues are similar to those with other forms of gene therapy, with the added complication that several different pieces need to be expressed by any one cell to have an effect (the targeting sequence and the molecular scissors). FARA is funding further early-stage research into the use of these tools in FA.

The FARA Advocate is brought to you by:

Contributors: Ron Bartek, Kyle Bryant, Felicia DeRosa, Jennifer Farmer, Erin Goerss, Matt Lafleur, Jane Larkindale, PhD, Angel Martin, PhD, Ann Musheno, Katie Schultz and Jamie Young.

Editor: Karen Smaalders

Design/ Layout: Crystal Ahern

Continued on next page

Genetic Therapies

FARA works closely with our partners in the gene therapy space. FARA has held meetings where scientists discussed the hurdles of gene therapy, and meetings involving companies and regulators to determine paths forward. FARA and its partners then worked cooperatively on trying to resolve some of those concerns. Patients have visited most of the groups working on FA therapies, to allow the companies to learn more about the disease and how it affects people, and have spoken at meetings including both regulators and companies about their preferences. FARA's goal is to make the development of such therapies for FA as smooth and rapid as possible.

Overall, genetic therapies offer significant potential as therapies for FA. They address the root cause of the disease (frataxin deficiency) in a long-lasting manner, potentially offering the hope of a cure. However, challenges remain in figuring out how to get them to produce the right amount of frataxin in all of the tissues that need it, figuring out how they perform over time (as there are challenges with administering such therapies more than once), and determining how well they may work at different stages of disease. Results of gene replacement in animal models look very positive, however, and we are very excited to see gene therapy development programs approaching the clinic for the first tests in patients.

Energy Ball

I fight not only for myself, but for my sister, my parents, for everyone else affected by FA and for those that could be. I fight for rights that everyone should have—a healthy heart, body and mind. I fight so that one day FA is simply part of our past. I fight for the cure for Friedreich's ataxia.

I am an FA Warrior.

-Alison Avery

The FARA Energy Ball: Knock Out FA

By Felicia DeRosa

(Clockwise starting left) Energy Ball Co-Founder Suzanne Avery, Auction Co-Chair Amy Hull, and committee members Lisa Lafita, Alison Avery and Laurel Avery pause for a souvenir photo in front of the Energy Ball backdrop.

The Tampa Community came together for the 9th consecutive year for the FARA Energy Ball—a sold out gala marked by its vibrant décor, warm welcome and sense of fun. In addition to being a great party complete with a delicious plated dinner, a competitive auction and energizing live entertainment, the event comes with a sense of purpose grounded in the fighting spirit of those who live with FA. The event was fittingly themed—Knock Out FA. Colorful boxing gloves adorned the centerpieces of each table, live auctioneer Scott Robertson stepped in center stage, fashioned to look like a boxing ring, with the well-known rally cry, "Let's Get Ready to Rumble," and event emcee Wendy Ryan emerged in her Team FARA boxing cape.

Later in the evening, Honorary Event Chairs Janell and Steve Griggs, CEO of the Tampa Bay Lightning, and Event Founders and Chairs Suzanne and Paul Avery, explained why they are so engaged in the fight to treat and cure FA. Suzanne shared, "With the passion of the group assembled here tonight and all the wonderful people all over the world working towards a cure of the FA disease, we will restore the energy to our children and loved ones, giving them the power of a full and rich life." This sentiment was further underscored in a video about a unique friendship in Tampa between two people living with FA, 26-year-old Sam Bridgman and 12-year old

Continued on next page

Gavin Lambert. Scott and Wendy teamed up to cheer on the crowd as guests raised $600,000 in a matter of minutes during the Fund a Cure, contributing significantly to an overall event total of $2 million! After an active live auction with bids on exclusive dining experiences and getaways, guests traded in their bidding paddles for dancing shoes to close out the night dancing to popular tunes from the live band, Blonde Ambition.

Everyone at FARA extends a sincere thank you to Event Coordinator Ava Forney, Auction Co-Chairs Amy Hull and Anna Alfano, the Event Chairs and the Planning Committee. FARA is also grateful for all of the many generous sponsors, auction and in-kind donors, bidders and attendees who made the evening so successful. In the wake of Hurricane Irma, the Tampa Community rallied despite personal adversity to help us fight for a cure. They are all truly FA Warriors.

(Clockwise starting left) FAmily photo featuring Anna Morrow, Christian Maugee, Gavin Lambert, Kate Walker, Michael Gehr, Shandra Trantham, Sam Hill, Sean Baumstark, Alison Avery, Erin Pieper, Jean Walsh, Sam Bridgman, Kyle Bryant, Isabel Maugee, and Laurel Avery.

Energy Ball Co-Founder and FARA Board Chairman Paul Avery and Event Coordinator Ava Forney celebrate a successful event at the end of the evening.

Thank You to Our Energy Ball Sponsors

The Avery Family Foundation

The Peter Crisp Family

REATA PHARMACEUTICALS

BLOOMIN' BRANDS: OUTBACK STEAKHOUSE, CARRABBA'S ITALIAN GRILL, BONEFISH GRILL, Fleming's PRIME STEAKHOUSE & WINE BAR

PLATINUM SPONSORS
Paul and Mary Jacobs
Horizon Pharma
Kurt and Amy Hull
Lightning Foundation
Southern Glazer's Wine & Spirits
Standard Meat
The Villages

DIAMOND SPONSORS
Agilis Biotherapeutics
Tom & Dede Bradley and Tom & Lauren Pepin
Jabil
The Brad and Nancy Rex Family
ShimSpine
World of Beer

GOLD SPONSORS
Bay Stage Live
The Corbett Family Charitable Foundation, Inc.
Shon and Sharyn Craig
Cydan
Corporate Creations
Cure FA Foundation
Eddie and Candy DeBartolo & Family
Health Insurance Innovations
The Hennecke Family Foundation
The Krutzer/ Fisher Family Trust
Jerry and Vickie Moyes
The Patron Spirits Company
PDQ
The Ritschel Family
Doug and Kathy Rothschild
Drs. Joseph Staffetti and Theresa Zesiewicz
Chris and Sandi Sullivan
Tampa Bay Lightning
Tampa General Hospital
Thornton Creek Motorcoach Resort
Tito's Handmade Vodka
USF Health

SILVER SPONSORS
Amalie Oil Company
Berman & Company
The Burrows Hill Foundation
Connie Gage, Realtor. The Toni Everett Co
Mike and Sheila Ingram/ El Dorado Holdings, Inc.
Mr. & Mrs. Joe & Teresa Kadow
Pfizer, Inc.
Raymond James Financial
Retrotope
RMC Property Group
Seminole Hard Rock Hotel & Casino
Trinchero Family Estates
USF Foundation
Vanguard Protex Global
Louis and Kelly Varsames
Verimed
Mr. W. Dan Wright
Dr. Paul and Mrs. Avery Zaritsky

TABLE SPONSORS
908 Group
A1 HR
Adrian Fernandez Insurance, Inc.
Frank and Anna Alfano
Berkeley Preparatory School
Beveridge Medical Associates, Inc. and Patel Brothers Inc.
Brown & Brown Insurance
Dr. Robert Dean
East Lake Pediatrics
Fintech
Florida Hospital of Tampa
Jason and Sarah Foglia
Friends of Sam
Mike and Lori Kosloske
Victor and Lisa Lafita
The Melting Pot Restaurants/ Front Burner Brands
Michael and Nicole Mezrah
Monin
Quarles & Brady, LLP
Gavin and Laura Southwell
SunTrust Banks
Tampa Bay Buccaneers

MEDIA SPONSORS
Clear Channel Outdoor
iHeart Media
Matlet/ Group-Central Florida Press
Tampa Bay Metro
Tampa Bay Times

Featured Scientist—Dr. Ben Deverman

By Matt LaFleur

"Please, just Ben."

The first thing I asked Dr. Ben Deverman is whether he preferred to be called "Dr. Ben" or "Dr. Deverman." He seemed relieved as he gave me his answer, and I knew that my job would be easy: I am lucky to introduce him in this newsletter.

One of the most exciting fields of scientific research is gene therapy, a term that leaves most of us suffering with FA feeling awe and wonder. If FA is, by definition, a genetic anomaly, then the science of correcting genes seems extremely promising for FA.

The exciting promise of gene therapy was delivered to us at the FA symposium by Ben Deverman, PhD, Senior Research Scientist, director of the CLOVER (CLARITY, Optogenetics and Vector Engineering Research) Center within the Beckman Institute at Caltech in Pasadena, CA:

"I run this center with Viviana Gradinaru who is an assistant professor and the center's principle investigator. We are focused on developing cutting edge technologies in the fields of gene therapy and gene transfer for scientific applications, tissue clearing (a way of making tissues transparent so you use microscopes to image complex biological processes in their intact 3D form - without making thin sections on slides) and optogenetics, which uses light-activated channels to manipulate the activity of neurons."

Reports on his findings did not lessen the promise of gene therapy as an exciting field in the search for a treatment of FA, but grounded the dream in a scientific reality, making it less of a fantasy. However, we know that gene therapy will not be a sudden "fix" for FA. "While gene therapy may not offer a cure for FA, if we can solve some of the remaining challenges, I'm hopeful that it will have a big positive impact on patients' lives," Ben explains.

Ben Deverman (middle) with FA families at rideATAXIA [+] SoCal.

The excitement of gene therapy's potential is, shockingly, not the focus of this article. The greatest strength of FARA as an organization is its way of humanizing people instead of only seeing them as means to an end. Those of us with FA are not patients or subjects, but Ambassadors; the FDA is not an obstacle to overcome in finding treatment, but a cherished group of allies; we are not merely strangers with only a common disorder, but a FAmily. Ben is not just a messenger of incredible scientific advances, but a noble and worthy person. And to miss that and only focus on his research may be typical, but it is ultimately incomplete. To see both the brilliance of his research and the strength of his character gives us a little more compassion, a little more well-roundedness. After all, it's the FARA way.

Ben likes to get away from the city in his free time, to clear his head. He enjoys taking his road bike out into the mountains near his home in Southern California. When he's not in the lab, he is often on his bike or hiking a trail, enjoying the scenery and the quiet. He also wishes he had time to do more traveling with his wife of seven years.

Ben says that he has always been interested in biology, so he pursued a career in molecular biology. After obtaining a PhD, he transitioned into the field of neuroscience and later became interested in figuring out how to transfer genes into the brain cells of living organisms using inactivated viruses. This process seemed to be exactly what was needed in the treatment of Friedreich's ataxia, a disorder he'd heard of but had never encountered. Ben recounts what led him to FARA:

Continued on next page

"We had success engineering viruses that could cross the blood brain barrier and deliver genes into the brain and spinal cord (the central nervous system). We then looked for (and found) a virus that would work in a similar way in the peripheral nervous system where the sensory neurons reside. The vector we developed that targeted sensory neurons also serendipitously worked well in heart muscle. Therefore, we thought we had a combination of two vectors that were ideal for delivering FXN. And based on this I wrote a grant to FARA to get funding to start the study."

After Ben wrote a grant for FARA, he received an email about the rideATAXIA event in SoCal. In a profound way, two of Ben's passions—his love of cycling and his scientific curiosity of the prospect of gene therapy—came together. Ben was hooked.

He formed a last-minute team of riders from his lab and met Kyle Bryant for the first time at the ride. FA became less of a random disorder and more of one with personal ramifications in Ben's eyes. He has continued working with FARA, traveling to Australia to give a presentation in Melbourne, going to the rideATAXIA in Philadelphia, and serving as a highlighted speaker at both the FARA Ambassador Training and FA Symposium in Philadelphia.

It's an amazing feeling, knowing that Ben is on our side. FA research is in the best and brightest hands. I want to thank Ben on behalf of FARA for everything he does. We are grateful and we appreciate you.

According to Ben, "The feeling of being on the cusp of contributing, in my own small way, to the possible treatment of a disease is highly motivating."

Your way is not small at all, Ben.

Partner of the Year and Friend & Ally Awards

FARA Announces Partner of the Year and Friend & Ally Awards

By Felicia DeRosa

The resources to advance research come from you, our FA community. The time, talent and financial support stem from your generosity and confidence that together we can make a difference. FARA celebrates this spirit of giving, resilience, and collaboration annually with our Partner of the Year and Friend and Ally Awards.

The Partner of the Year award honors an individual or company whose contributions significantly increase FARA's capacity to carry out our mission to treat and cure FA. Often Partner of the Year recipients lead by example, engaging both their professional and personal communities in the cause. The Friend and Ally Award celebrates the commitment of volunteers who work constantly (and many times unrecognized) by our sides. The recipients of this award are the people who show up, who give repeatedly without needing to be asked.

FARA is proud to present this year's Partner of the Year Award to the Honorary Chairs of the Energy Ball for the past three years, **Steve and Janell Griggs**. Steve and Janell were first introduced to FARA through Steve's predecessor at Tampa Bay Lightning, Tod Leiweke. Steve has often remarked that when he first agreed to chair the Energy Ball, it was to honor Tod's commitment to the cause. Over a short time, as they got to know local people living with FA, Steve and Janell's connection to the cause became more personal. Steve met his friends with FA on the bike trail for a Saturday ride. Janell met members of the Energy Ball committee for coffee. Both pledged to support as much as possible—and that they did with sponsorship of the Energy Ball, as hosts of the Patron Party, and through donation of Lightning trips, memorabilia to the auction, and an entire suite for the Santana concert for Fund a Cure giving. With generosity and leadership, Steve and Janell enhanced the level of the Energy Ball

Continued on next page

Alison Avery with FARA's Partner of the Year honorees Steve and Janell Griggs.

Tim Block's Outback Southeast Region team celebrates their strong fundraising for FARA.

event and its capacity for fundraising and brought important supporters, new awareness and critical resources to research. We are grateful to call them partners in this fight to cure FA.

The two honorees for the Friend and Ally Award are **Charlotte Pizzo** and **Tim Block** of the Outback Southeast Region. Charlotte has been a key teammate in the success of rideATAXIA Philly. For nine years, Charlotte coordinated the food from Outback Steakhouse, Bonefish Grill and Carrabba's Italian Grill at the ride. If you've ever attended rideATAXIA Philly, you know the food is the main event. Even when staff and leadership changed in her Outback team, Charlotte was the constant who ensured we maintained the best post ride food. In addition to coordinating the huge food effort, Charlotte has executed many other tasks like making early morning coffee and hot cocoa; transporting grills to the event site; and securing donations from key sponsors like Hank's Root Beer and Coastal Sunbelt Produce. Charlotte does this all with efficiency and a smile, without seeking recognition, just as a true friend.

Tim Block and the Outback Southeast Region maintain an incredible grassroots fundraising effort spanning the Outback Steakhouse restaurants throughout the Carolinas. For years, Tim has led the region in organizing events such as golf tournaments and 5K runs. Tim and his team have fundraised and hosted events independently, devoting a significant amount of time and effort to the cause. Each fall, Tim and his team send the proceeds from their fundraising, upwards of $50,000 annually, and they do all of this without ever needing to be asked. Tim is the kind of friend and ally who continues to give where he sees the need. Thank you to Steve, Janell, Charlotte, Tim and all of our generous partners, friends and allies.

Ataxian Athlete Initiative

The AAI has provided adaptive cycling equipment for 42 individuals since 2009. In 2017, the AAI funded 9 grants to athletes from 7 states and 2 countries including: Ashlea Smith of Aliquippa, PA, Jacob Tompkins of Tivoli, NY, Joni Moore of Superior, WI, Justin Hernandez of Graham, TX, Katie Hook of LaPorte, IN, Kevin Henry of Jackson, MO, Natchez Hanson of Palm Coast, Fl and Savvas Nicolaou of Limasoll, Cypress.

Visit curefa.org/aai for more information and to view more photos of past recipients. The next application cycle begins in Spring 2018.

Team FARA: Medtronic Twin Cities Marathon and 10 Mile

By Ann Musheno

In a marathon effort steered by team captain and FA parent Tom Batta, Team FARA raised more than $70,000 as part of the Medtronic Twin Cities Marathon and 10 Mile. Twenty-one team members participated in the October 1, 2017 event in St. Paul and Minneapolis, MN, working together to create one of the highest grossing Team FARA events ever!

This is not Tom's first time running a marathon for Team FARA; he raised over $20,000 in the 2016 NYC Marathon. Though initially hesitant to start another fundraising campaign, he invited his running club, the Watertown Run Club, to participate. After seeing the club rally around the cause, excited to both run and fundraise, he decided that "I can't let everybody else put in all the work and effort while I just sit on the sidelines."

To get the word out to larger communities in Watertown and Sioux Falls, SD, the team shared their story three times on local radio as well in the local newspaper and TV news. Some team members used other tactics to fundraise including a LuLaRoe party and a jewelry raffle. Team member Owen McElroy even offered his highest donor the opportunity to shave anything into his head. The winner was a jewelry store owner, so Owen ultimately ran the race with diamonds as well as FARA shaved onto his head!

Dan Brendtro, FA parent and one of the 15 marathon runners, raised nearly $29,000 – surpassing his goal to raise $1,000 per mile. He used Facebook as his primary fundraising tool, posting daily for 13 days. In addition, he called, emailed, and texted 30 people whom he thought would give with some prompting. To thank his donors, his family threw an appreciation party for them.

Tom and Dan both intend to keep fundraising for FARA – whether through more marathons or community-based fundraisers. Tom knows that with FA's status as a rare disease, financial support from

Members of Team FARA Medtronic Twin Cities Marathon and 10 mile.

FAmilies alone will not be enough to get us to the ultimate finish line – a cure for FA.

Team FARA is made up of people around the globe who participate in endurance events on behalf of FARA with the goal of raising awareness and funds for FA research. In addition to marathons and 10Ks, past Team FARA members have registered as individuals and groups in triathlons, ironman competitions, bike tours and 5K runs.

Team FARA is part of FARA's Grassroots Program, which for the past three years, has exceeded its $1 million annual fundraising goal set by Mission 1 Million. This program is once again on track to reach another $1 million in 2017. To learn more about joining Team FARA visit **curefa.org/team-fara**. To learn about other grassroots events occurring across the country or how you can host your own grassroots fundraiser, visit **curefa.org/grassroots.** Together we will cure FA!

Advancing Research: A Community Effort

By Katie Schultz

Katie Schultz joined the FARA team, as the Patient Engagement Director, in early February. Katie serves as a liaison between the patient community and other FARA stakeholders such as the pharmaceutical industry, academic scientists, and governing bodies to promote understanding of the FA condition and incorporation of FA families in research.

FA patients and caregivers, along with academic researchers and industry partners, are interdependent—each providing critically important information about the puzzle that is Friedreich's ataxia. Together, their contributions are helping to advance the science towards approval of meaningful treatments.

The FA community is actively involved in visiting academic researchers to better understand the early-stage studies occurring in their labs, and to educate researchers about life with FA. For example, a patient/caregiver group visited Dr. David Corey's lab at Dallas Southwest Medical Center and Dr. Marek Napierala's lab at the University of Alabama, Birmingham. Families learned how the skin cells of FA patients gathered at Dr. Lynch's lab at CHOP are being used with oligonucleotides to block the genetic defect, resulting in increased frataxin in cells in culture. These same cells are being reprogrammed into cardiac and brain cells so that differences in frataxin protein expression can be studied. They heard about making a mouse model with the G130V mutation so that potential drugs could be tested and might one day be used in human clinical trials. In turn, the researchers had the opportunity to meet people living with FA to understand how their research impacts people.

To move into human clinical trials, industry must be willing to commit the resources needed to leverage and expand upon the basic knowledge gained from the lab. Fortunately, several companies are doing just this, but their success depends on a strong relationship with the patient community. Many FA patients have participated on panels to share the impact that FA has had on their lives, as well as their perspective about meaningful treatments. This input has been

The Dagley and Henry Families with Dr. David Corey and his team.

critical to how our partners think about FA and the design of clinical trials. In addition, they recognize the importance of educating their employees about FA. Earlier this year, members of the FA community met with the senior leadership at Adverum Biotechnologies. In addition to touring Adverum's facility and attending a screening of The Ataxian together, the group discussed what it is like to live with FA as well as Adverum's approach to gene therapy.

Finally, the collaborative efforts among the patient community, academia, and industry to develop meaningful FA therapies would not be as fruitful without the continued strong support of regulators at the Food and Drug Administration (see President's Message about the Patient Focused Drug Development Meeting).

The Penston, Konanz, Bennett, and Bryant families during their visit to Adverum Biotechnologies.

The FAmily takes a moment to pose at the 5th Annual Mother's Day 5K Race for Christina on May 14 in Richboro, PA.

The Burrows Hill Foundation to Fight Friedreich's Ataxia took a really big swing at FA during their "Night to Fight FA" on April 1 in Annapolis, MD to benefit research and the AAI at FARA.

Anna Morrow (Center) with friends at the Morrow Family's 2nd "Hope for ToMOR-ROW event" in Baltimore, MD on March 24.

The Myers Family at the Slim's Journey FARA 5K Run/Walk on September 23 in Warrenton, MO.

Held up by her parents, Wendi and Matt, Alison Price (3rd from left) shows off her super hero gear at the Super Hero 1 Mile & 5K Run on October 28 in Wake Forest, NC.

Teams try their best to be the champion at Toss Out FA, a corn hole tournament fundraiser held on September 16, 2017 in Peoria, IL.

Francine, Pete, Eilish, and Brendan Welsh pose for a family picture at the 11th Annual Welsh Bash in the Backyard on July 22, in Harrisburg, PA.

Jack DeWitt and Kyle Bryant (front) with FAmily at a screening of The Ataxian organized by the DeWitt Family on October 8 in Howell, MI.

New Jersey FAmily gathers for a photo at the N.J. Seaside Stride in Seaside Park, NJ on May 20.

Hannah Stacks begins the wiffle ball tournament at the 8th Annual Swing Away at FA in Dawsonville, GA on October 21.

Kyle Bryant and Kate Walker show off one of the displays at The Ataxian screening organized by the Walker Family in Ruston, LA on August 19.

Students enjoy some friendly competition at one of the Lincoln School's Hoops for a Cure Nights held in Palantine, IL in February.

Kyle Bryant and Matt LaFleur show off matching team shirts ahead of a screening of The Ataxian in Lafayette, LA on August 20.

Nadine Belliveau and Laurel Avery enjoy one of World of Beer's famous pretzels at the Pull for a Cure on February 11 in Land O lakes, FL.

Alex Fielding speaks before the Race for Matt & Grace on September 23 in Providence, RI.

The FAmily at FA Woodstock in July. FA Woodstock is three days of camping, swimming and music in LaPorte, IN hosted by the Hook Family for people with FA and their families.

FAmily and FARA Staff gather at the Fuzzy Buzzy Golf Tournament on September 10 in Windham, NH.

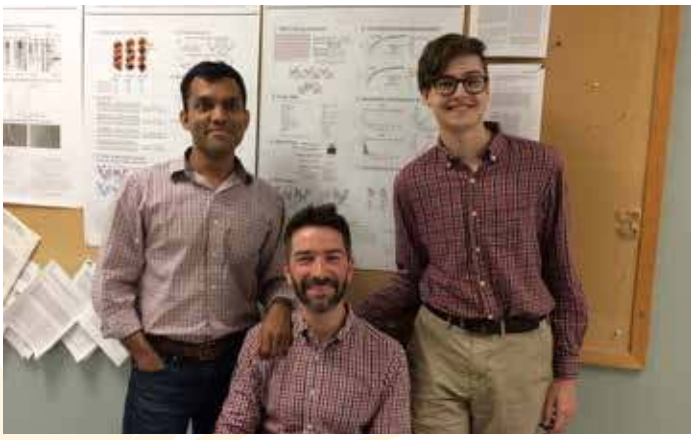
Surrounded by a mountain of prizes, Josh Lamascus speaks to attendees at the Century 21 Charity Golf Tournament on September 21 in Fontana, CA.

Kevin & Dawn Lambert and Donald & Ceron Buti enjoy the success of their FArm to Table event in Plant City, FL.

Golfers added some flair to their outfits with grass skirts and feather boas at the 35th Annual Claxton Classic Invitational Golf Tournament on July 21 in Glenn Dale, MD.

Anna Gordon sits on her throne surrounded by friends at the #Anna'sArmy Mermaid Masquerade on April 1 in Vienna, WV.

The McDonnell Music Festival celebrated their 10th Annual event on September 16 in Queensbury, NY. Sadly, it was the first without Terry McDonnell, who passed away in May after a long battle with cancer. This photo features Terry (back row, 2nd from left), her son Dylan McDonnell (front row, 1st from left), and husband David McDonnel (back row, 1st from left), surrounded by supporters at their music festival several years ago.



Yogesh Chutake, Kyle Bryant and Michael Gehr show off their coordinated outfits during a visit to Sanjay Bidichandani's lab at Oklahoma University.

After a long day of golf and clay shooting, FAmily presents the final check at the Rocky Mountain Bird & Birdie held on September 22 in Brighton, CO.

FARA
533 W Uwchlan Ave
Downingtown, PA 19335

WINTER 2017 - 2018

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance

This Issue:

FARA Friedreich's Ataxia Research Alliance

CUREFA.ORG