



FAMILY Newsletter

Fall 2019

A semi-annual publication of the Fanconi Anemia Research Fund

issue
66

Introducing the

FAdult COUNCIL



In late 2017, for the first time ever, there were more adults living with Fanconi anemia than children (according to FARF's registry). This reflects an emerging population of adults that was not present even just a decade ago. This growth in the adult population could be attributed to the dramatic increase in survival following stem cell transplants over the last 20 years. As a result, more children are reaching adulthood.

In addition to the normal challenges one faces when becoming an adult, like working and learning to live independently, adults with FA are confronted with a unique

set of challenges. Some of these include transitioning complex healthcare from parents to the individual, the lack of experienced FA clinicians who specialize in adult care, issues with fertility, and the increased likelihood to develop solid tumors.

To respond to these challenges, and to better understand other issues facing this population, earlier this year FARF formed an FA Adult Council, or FAdult Council. Currently comprised of seven adults with FA, the group is "a permanent advisory council to provide ongoing support to the FARF staff and board of directors by providing informed input as



**JASMINE
BENNETSEN**

Age: 27

Residence: Florida

Transplant Status: Minnesota 2019

Occupation: Apple, photographer



**MATTHEW
PEARL**

Age: 22

Residence: Park City, Utah

Transplant Status: Minnesota 2006

Occupation: Recreation Instructor at
National Ability Center



**ANGELA
BEDOYA**

Age: 26

Residence: Minneapolis, Minnesota

Transplant Status: Minnesota 2010

Occupation: Graduate student at the
University of Minnesota

Introducing the FAdult Council

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FARF plans new activities or develops policies and procedures related to FAdults™ (FAdult Council purpose statement).

The council determined their top objectives are:

- To provide advice and ongoing support
- To help people with FA have a voice
- To empower all adults with FA as one community

Each year, the council meets three times in person, three times via webcam, and on other occasions as needed. In their first few meetings, members outlined the role they seek to fulfill as a council:

- Act as a bridge between other FAdults and FARF
- Support other FAdults
- Encourage and inspire kids who have FA by setting examples for success and reminding them they are not alone
- Provide counsel and feedback to the FARF board of directors and staff
- Interact directly with FA families to offer advice or share experiences
- Help FARF create a guidebook specific to adults living with FA
- Serve as FARF Ambassadors at events, meetings, fundraisers, and conferences.

At the 2019 FA Family Meeting in June, five council members held a panel discussion for parents as well as a support group for teens and kids with FA. The council was also instrumental in developing the 2019 Meeting for Adults with FA in September.

If you're interested in getting in touch with any of the council members, please let us know (info@fanconi.org). ■

“We are in this together, and there is power in numbers.”



**AMELIA
HAWKSHAW**

Age: 26

Residence: Sydney, Australia

Transplant Status: No transplant

Occupation: Youth worker for Fusion



**MARY-BETH
JOHNSON**

Age: 27

Residence: Chicago, Illinois

Transplant Status: No transplant

Occupation: Private chef



**DUNCAN
NUNES**

Age: 23

Residence: Boston, Massachusetts

Transplant Status: No transplant

Occupation: Scientist at Novartis Institute for Medical Research



**JACK
TIMPERLEY**

Age: 20

Residence: Park Ridge, Illinois

Transplant Status: Minnesota 2002

Occupation: Student of business and philosophy at Northeastern Illinois University

2019 FAdult Council Members

Jasmine Bennetsen, *co-chair*
Matthew Pearl, *co-chair*
Angela Bedoya
Amelia Hawkshaw

Mary-Beth Johnson
Duncan Nunes
Jack Timperley



Scientist Spotlight

Name: Paula Río

Institution: Centro de Investigaciones Energéticas Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER-ISCI) and Instituto de Investigación Sanitaria Fundación Jiménez Díaz (IIS-FJD/UAM)

Area of expertise: Gene therapy and gene editing

My work: is focused on gene therapy and gene editing strategies to correct hematopoietic stem cells in Fanconi anemia patients. Led by Dr. Juan Bueren and together with my dear friend Dr. Susana Navarro, we work on the characterization and follow up of the stem cells from FA patients in Spain. In collaboration with Dr. Julian Sevilla and other members of the FA network in Spain, we are conducting a phase I/II gene therapy trial to correct hematopoietic progenitors from FA patients using lentiviral vectors (see p. 4-5). Together with Rocket Pharma we are participating in a new phase II gene therapy trial. I am also working on another project with my own lab, which is more focused on gene editing strategies to correct hematopoietic stem cells from FA patients. When I started to work on gene therapy, gene editing was like a dream. Now my dream is that in the future we can conduct *in vivo* gene correction in FA hematopoietic stem cells.

What motivates me to work on FA: The patients and the families; that is clear for me. When I was an undergraduate student, I realized that I would like to work in genetic engineering to correct inherited disorders, but I could never even have dreamed of working on a project like this. I did my PhD in Dr. Bueren's lab working on the characterization of the *Fanca* knock-out mouse model and the first therapeutic vectors to correct mouse hematopoietic stem cells. At that time, Dr. Bueren started to collaborate with clinicians, patients and researchers and he realized that there were many things to do for the patients, and together we established an FA network in Spain. I never thought about

meeting a patient with FA when I was initially working on the mouse model. It is difficult to explain what you feel. You are working really hard to get results for your project but suddenly you meet patients and families, and you see that they really trust you and what you are doing. You find the most important reason to work and go on, repeat and try again, no matter how many times. This is what motivates me and our team the most. When new students in the lab meet with patients and families, their way of thinking about work changes completely. It is not a job any more...it is a commitment.

When I'm not in the lab, you could find me: with the people that I love. My family and my friends are my life! That is my way of charging my batteries to keep going. I love having a good time together doing simple things: talking, going for a walk, dinner, cinema...overall, just sharing time with them. There are also times you won't be able to find me because I also like traveling!

Anything else you want FA families to know? I admire you. I have learned many things from all of you. I am really grateful for the opportunity you have given us to meet such strong people and to encourage us to go on working. I feel really lucky to have found this incredible community of people. Every time we meet people with FA and families, it is a new burst of energy for us to go on working on this disease. You all can be completely sure that the Spanish team will continue to do everything possible to improve your quality of life. ■



Paula's gene editing team



Paula's gene therapy team



Gene Therapy in Fanconi Anemia: An Update

Why gene therapy?

We know that Fanconi anemia (FA) is caused by defects in any one of 23 different genes called “FANC” genes. If we could somehow repair or replace these defective FANC genes, we could treat or prevent FA disease. Gene therapy is an approach to do just this. Gene therapy addresses the root cause of inherited diseases like FA by enabling cells to express a copy of a normal FA gene, thus allowing them to produce proteins necessary for health.

The cells we want to fix are unique types of cells in the bone marrow called stem cells. Stem cells have the ability to generate all other types of blood cells in the body, like white cells, red cells, and platelets. If we can fix the defect in stem cells, then all blood cells that are derived from a stem cell are also fixed. This should restore normal function to diseased stem cells and prevent bone marrow failure (BMF). FA commonly presents first with BMF, so fixing bone marrow cells to prevent this failure is crucial.

The current standard of care for BMF for people with FA is bone marrow transplant using hematopoietic stem cells from healthy donors. Thanks to advancements in transplant protocols, there is now a 90% chance of survival. Despite this relative success, however, transplants require the use of highly toxic chemotherapy regimens and in some instances total body irradiation to deplete failing FA stem cells before patients can receive healthy donor stem cells. These toxic regimens lead to whole-body DNA damage and increase the risk for development of graft-versus-host disease (GvHD) and squamous cell cancers in an already at-risk population. That’s where gene therapy comes in.

How does it work?

Blood and bone marrow contain all types of cells, including rare stem cells. In gene therapy, stem cells are first mobilized from the bone marrow and then extracted from the blood. Stem cells are then separated from other blood cells using a purification device. This procedure is called apheresis. A

fully functional, normal FA gene is then added to the DNA of FA stem cells to create normal, non-diseased cells. These cells are then infused into the patient. The hope is that these repaired stem cells will populate the bone marrow and prevent bone marrow failure and leukemia.

Where are we now?

Efforts to use genetically modified cells in clinical trials as a way to bypass the need for bone marrow transplants for people with FA have been unsuccessful in the past. This recently changed with the first ever published report demonstrating the possibility of engrafting corrected stem cells in FA patients who did not receive any previous conditioning chemotherapy (in contrast to bone marrow transplants). The first results of this gene therapy trial were published by Drs. Paula Río, Susana Navarro and co-workers in September of 2019 ([clinicaltrials.gov NCT03157804](https://clinicaltrials.gov/NCT03157804)). Dr. Juan Bueren, the coordinator for the study, and his team recently reported in the journal, *Nature Medicine*, on the success of a gene therapy trial for people with complementation group FA-A. The phase I/II clinical trial was conducted at Foundation of the Hospital del Niño Jesús in Madrid, under the direction of Dr. Julián Sevilla, in close collaboration with the Center for Energy Environment and Technology (CIEMAT), the Center for Biomedical Network Research on Rare Diseases (CIBERER), and the Institute of Health Research of the Jiménez Díaz Foundation (IIS-FJD), among many other partners.

The clinical trial was initiated in 2016 for FA patients with *FANCA* mutations who had bone marrow failure at the start of the study. The goal of the trial was to use autologous (from the patient) stem cells that were mobilized from bone marrow and extracted from peripheral blood (apheresis). The extracted stem cells were then purified, transduced with a lentiviral vector carrying the wild type (normal) *FANCA* gene. Patients were then infused with the gene-corrected cells. Nine patients between the ages of 2-6 years were

“ After almost three years of treatment, the clinical investigators report that more than half of the blood cells from one of the treated patients are “healthy” cells that have the corrected FANCA gene. Importantly, no adverse events have been reported in any of the patients. ”

infused with either frozen or fresh FANCA gene-corrected stem cells without the use of harmful conditioning agents. This clinical study varied from past gene therapy trials for FA patients both with respect to the agents used to extract cells from the bone marrow, the therapeutic vector and the conditions used to transduce the stem cells, including a short transduction period (time used to add the correct gene to stem cells while they were outside of the patient).

In the *Nature Medicine* publication, the clinical trial team reported on outcomes for four patients 18-30 months after infusion with the genetically corrected stem cells. The results indicate that peripheral blood cells expressing the corrected FANCA gene were detected in all four patients six months after infusion; and, remarkably, the number of blood cells with the corrected gene then increased in all patients who were studied over 18 to 30 months after treatment.

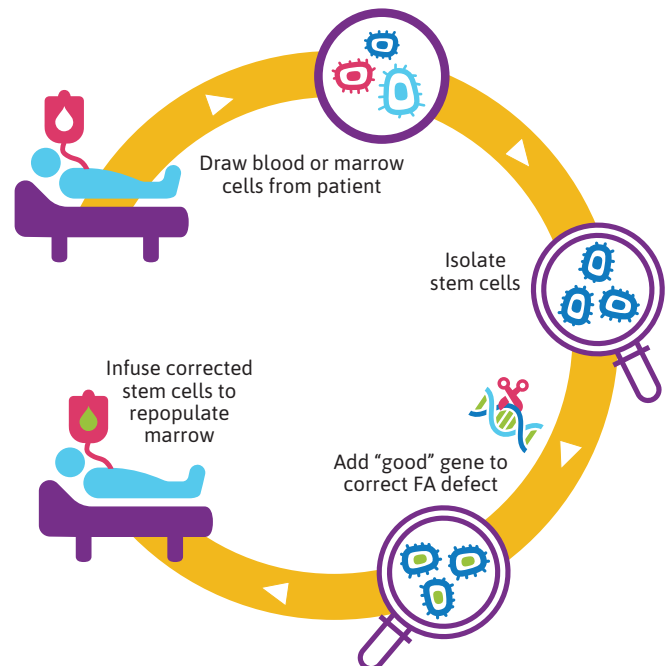
These results indicate that the corrected stem cells engrafted patient bone marrow and that blood cells derived from the stem cells are thriving and maintaining expression of the normal FANCA gene. Functional laboratory studies also demonstrated that the normal cells were expressing a functional FANCA protein, as the cells were resistant to DNA damaging agents, such as mitomycin-C, and had decreased chromosomal breaks after exposure to diepoxybutane (DEB). After almost three years of treatment, the clinical investigators report that more than half of the blood cells from one of the treated patients are “healthy” cells that express the correct FANCA gene. Importantly, no adverse events have been reported in any of the patients.

What’s next?

Rocket Pharmaceuticals, Inc., has now expanded and sponsored the clinical trial to a new phase I trial (clinicaltrials.gov #NCT03814408) that was initiated at Stanford University under the direction of principal investigator Dr. Sandeep Soni. This trial enrolled two patients aged five and six years who are in complementation group FA-A, and is no longer recruiting patients. The goal of the study is to determine safety and preliminary efficacy of autologous gene correction for the FANCA gene in hematopoietic stem cells without the use of harmful conditioning agents.

Similar to the gene therapy trial in Spain, these patients underwent stem cell mobilization, apheresis, transduction with lentiviral vectors to correct the stem cells, and transfusion of the stem cells back into each patient. The differences between the new phase I study at Stanford and the study in Spain include the use of commercial manufacturing processes to optimize the lentiviral transduction procedure and no freezing of stem cells prior to transfusion back into the patient. Both patients will be followed for three years post-infusion of the corrected cells to monitor for safety and early evidence of efficacy. Initial results from these two patients may be available by late 2019.

The investigative team at Stanford is also currently developing a phase II study for gene therapy in FA-A patients with enrollment expected to open in the coming months. In addition, a highly similar phase II study, also sponsored by Rocket Pharmaceuticals, is also underway at Hospital Niño Jesús in Madrid with initial enrollment planned for the autumn of 2019. ■



The Gene Therapy Process for Fanconi Anemia

Current **CLINICAL TRIALS** and **RESEARCH OPPORTUNITIES**

New treatments and therapies for people with FA are not possible without research. Listed below are current clinical trials and research opportunities available.

Visit the links listed to learn more about eligibility and protocol descriptions. If you're interested in participating in a clinical trial, scholarships are available from FARF in order to help offset the cost of transportation and housing.

SOLID TUMORS

Tumor Testing Available to Help Identify Therapy Options

If you or your loved one develops a tumor and would like guidance about potential personalized therapies, please contact FARF team members Dr. Sudhir Borgonha or Dr. Isis Sroka to help you navigate options and next steps. FARF has a relationship with the Knight Cancer Institute at Oregon Health & Science University to make sequencing available to Fanconi anemia patients who develop a malignancy. The Knight Diagnostic Laboratories (KDL) specialize in molecular diagnostic testing that may lead to targeted drug therapy options for patients based on the identification of DNA mutations in cancer samples.

Dr. Sudhir Borgonha, FARF Translational Science Director | 541.687.4658 | sudhir@fanconi.org

Dr. Isis Sroka, FARF Director of Scientific Operations | 541.687.4658 | isis@fanconi.org

Reducing the Burden of Squamous Cell Carcinoma

New methods are being developed to detect very early signs of oral cancer in simple, noninvasive ways. One of these methods involves brushing samples from the mouth with soft brushes and testing these samples for abnormal cells. This research study has three goals: to determine if this test would be helpful for people with FA, to learn more about molecules in the saliva that could indicate the presence of cancer, and to improve research collaborations in FA.

Contact: FARF | 541.687.4658 | suzanne@fanconi.org

Study of Pembrolizumab (MK-3475) for High Risk Oral Intra-Epithelial Neoplasias

M.D. Anderson Cancer Center, Houston, TX | currently recruiting participants

The goal of this clinical research study is to compare pembrolizumab to standard of care observation (no treatment) in controlling oral pre-malignant lesions. Pembrolizumab is FDA approved and commercially available for the treatment of

certain types of melanoma and non-small cell lung cancer. It is currently being used for research purposes in head and neck cancer. FA patients who have not had a transplant and who have a history of oral lesions may be eligible to apply for this trial. <https://clinicaltrials.gov/ct2/show/NCT02882282>

Contact: Renata Ferrarotto | 713-792-6363 | CR_Study_Registration@mdanderson.org

A Study of Prexasertib in Patients with Solid Tumors with Replicative Stress or Homologous Repair Deficiency

Dana-Farber Cancer Institute, Boston MA | currently recruiting participants

This is a research study of a checkpoint kinase 1 (CHK1) inhibitor as a possible treatment for advanced solid tumors that harbor genetic alterations in the homologous repair (HR) pathway or with genetic alterations that indicate replication stress. <https://clinicaltrials.gov/ct2/show/NCT02873975>

Contact: Geoffrey Shapiro | 617-632-4942 | Geoffrey_Shapiro@dfci.harvard.edu

Quercetin Chemoprevention for Squamous Cell Carcinoma in Patients with FA

Cincinnati Children's Hospital Medical Center, Cincinnati, OH | currently recruiting participants

In the lab, quercetin, a natural antioxidant, kills tumor cells in FA head and neck squamous cell carcinoma (SCC) cell lines and also prevents development of SCC tumors in non-FA mice.

Based on these strong and promising data this study will look at the beneficial effects of oral quercetin treatment for 2 years, in post-transplant patients with FA. It is hoped that treatment with quercetin will result in decreased oxidative stress and ongoing DNA damage of the mucosa, leading to the prevention of, or at least delay the development of squamous cell carcinoma. <https://clinicaltrials.gov/ct2/show/NCT03476330>

Contact: Stephanie Edwards | 513-636-9292 | StephanieL.Edwards@cchmc.org

BONE MARROW FAILURE

Eltrombopag for People with Fanconi Anemia

National Heart, Lung, and Blood Institute (NHLBI), Bethesda, MD | currently recruiting participants

Objective: To find out if a new drug, eltrombopag, is effective in people with Fanconi anemia and to know how long the drug needs to be given to improve blood counts. <https://clinicaltrials.gov/ct2/show/NCT03206086>

Contact: Evette Barranta | 301-827-4421 | barrantae@mail.nih.gov

Quercetin in Children with Fanconi Anemia; a Pilot Study

Children's Hospital Medical Center, Cincinnati, OH | currently recruiting participants (pre-transplant)

This is a pilot study aiming to assess feasibility, toxicity and pharmacokinetics of oral quercetin (a dietary supplement) therapy in patients with FA and is a first step towards a clinical study of the efficacy of quercetin therapy in delaying progression of bone marrow failure in FA. <https://clinicaltrials.gov/ct2/show/NCT01720147>

Contact: Stephanie Edwards | 513-636-9292 | stephanieL.edwards@cchmc.org

Sara Loveless | 513-803-7656 | Sara.Loveless@cchmc.org

Pilot Study of Metformin for Patients with Fanconi Anemia

Boston Children's Hospital, Boston, MA | currently recruiting participants

Preclinical studies from Oregon Health & Science University (OHSU) suggest that metformin may improve blood counts in an FA animal model. This clinical trial is being conducted to determine if metformin improves blood counts in people with FA. The study also looks at the effects of metformin on DNA damage and aldehydes. You may be eligible for this study if you have FA and low blood counts, are between the ages of 6-35 years, and have not had a bone marrow transplant. As a participant in this study, you will be provided with metformin for 6 months and your blood counts, other laboratory tests, and clinical symptoms will be monitored while you are in the study. There are 2 required visits to Boston Children's Hospital and compensation for reasonable travel expenses is provided. <https://clinicaltrials.gov/ct2/show/NCT03398824>

Contact: Ashley E Kuniholm | 617-355-6513 | ashley.kuniholm@childrens.harvard.edu

Jacob Cotton | 617-919-4227 | jacob.cotton@childrens.harvard.edu

GENE THERAPY

A Clinical Trial to Evaluate the Safety of RP-L102 in Pediatric Subjects With Fanconi Anemia Subtype A

Stanford University and Lucille Packard Children's Hospital, Stanford, CA | active; not currently recruiting

The objective of this study is to assess the therapeutic safety and preliminary efficacy of a hematopoietic cell-based gene therapy consisting of autologous CD34+ enriched cells transduced with a lentiviral vector carrying the FANCA gene in subjects with Fanconi anemia subtype A (FA-A). Blood stem cells collected from an FA-A patient are genetically

modified to introduce an intact copy of the FANCA gene using a virus that has been changed in the laboratory so that it cannot grow or spread to cause an infection. The genetically modified cells are then returned back into the patient. <https://clinicaltrials.gov/ct2/show/NCT03814408>

Contact: Sandeep Soni, MD | 650-725-9250 | ssoni1@stanford.edu
Agnieszka Czechowicz, MD, PhD | 650-497-2218 | aneeshka@stanford.edu

Lentiviral-mediated Gene Therapy of Fanconi Anemia Patients Subtype A (FANCOLEN-1)

Hospital Infantil Universitario Niño Jesús, Madrid, Spain & Hospital Vall d'Hebron, Barcelona, Spain | active; not currently recruiting

This is an open clinical trial to evaluate the safety and efficacy of a hematopoietic gene therapy procedure with an orphan drug consisting of a lentiviral vector carrying the FANCA gene for patients with Fanconi anemia of subtype A. <https://clinicaltrials.gov/ct2/show/NCT03157804>

Contact: Julian Sevilla | +34 915035938 | julian.sevilla@salud.madrid.org

NATURAL HISTORY

Cancer in Inherited Bone Marrow Failure Syndromes

National Cancer Institute (NCI), Bethesda, MD | currently recruiting participants

This is a study to provide information regarding cancer rates and types in inherited bone marrow failure syndromes (IBMFS), including Fanconi anemia. It is a natural history study, with questionnaires, clinical evaluations, clinical and research laboratory tests, review of medical records, and cancer surveillance. <https://clinicaltrials.gov/ct2/show/NCT00027274>

Contact: Blanche P. Alter | 240-276-7239 | alterb@mail.nih.gov

Natural History of FANCD1/BRCA2

National Cancer Institute (NCI), Bethesda, MD

We previously determined that published cases with two mutated FANCD1/BRCA2 genes appeared to have a very high risk of cancer before age 6. We are now aware of individuals with these mutations who are much older and have not had cancer. In order to determine the natural history of patients with FA associated with mutations in FANCD1/BRCA2, we have created a subgroup within the National Cancer Institute study of Cancer in Inherited Bone Marrow Failure Syndromes (above). <http://www.marowfailure.cancer.gov/>

Contact: IBMFS Study Team | 1-800-518-8474 | NCI.IBMFS@westat.com

TRIALS vs. TREATMENT

It is critical to understand the difference between medical treatments and clinical trials. A medical treatment is a regimen specific to an individual patient and his/her condition, administered by doctors. A trial tests a potential drug, procedure, or medical device in people. Participants in trials play an integral role in determining the safety and efficacy of drugs or procedures. It is important to remember that clinical trials are meant for research, not to administer proven medical care. ■



Fanconi Anemia Research Fund and NORD Launch Natural History Study of Fanconi Anemia

The research study is open to participants worldwide to advance understanding and treatments for the rare disease Fanconi anemia, diagnosed and confirmed through a combination of clinical findings and genetic analysis.

This September, the Fanconi Anemia Research Fund (FARF) and the National Organization for Rare Disorders (NORD) launched the largest-ever study to research Fanconi anemia (FA), a patient-driven natural history registry that will result in a wide variety of clinical findings.

The new study, 'An observational study in Fanconi Anemia', creates a platform for patients around the world to share information about their experiences living with Fanconi anemia. Its purpose is to build an international resource to be used by scientists in future research and clinicians in future treatment.

What is the Fanconi Anemia Registry?

The Fanconi Anemia Registry is a natural history study that consists of electronic surveys to collect information about the patient experience and disease progression. It is a versatile online system that securely collects and stores data for medical research. Patients, or their caregivers or guardians, can enter information from anywhere in the world. Registry participants can complete surveys about their own disease experiences in addition to learning about other participants' experiences by viewing anonymized aggregated survey data.

The Fanconi Anemia Registry is a powerful opportunity for individuals with FA and their family members to contribute directly to research that will enhance our understanding of the disease, thus facilitating the development of new diagnostic and treatment options. Participation is especially vital given the rarity of FA – every patient experience is a unique and invaluable part of the natural history of FA.

Who runs the registry?

FARF is launching the registry in collaboration with the National Organization for Rare Disorders (NORD), an

independent charity that built its natural history study platform as part of its mission to help identify and treat 7,000 rare diseases. FARF is a member of NORD and the organizations work together to eliminate the challenges that rare disease patients face. FARF is the custodian of the registry and the data contained in the registry and will be your main point of contact throughout your participation in the registry study. Each patient is the sole owner of his/her own data.

Does it cost?

Participation in the Fanconi Anemia Registry is free and voluntary, and participants may withdraw at any time.

What about privacy?

The Fanconi Anemia Research Fund will ensure that data privacy and confidentiality are strictly maintained. Only authorized personnel at FARF will have access to the individual data. If data is requested for research purposes, it will always be provided in a de-identified manner. The data is made anonymous and stored securely in an online portal.

Who is the registry for?

Any patient with a diagnosis of Fanconi anemia may join this registry.

If I'm interested, what should I do?

Contact Suzanne Planck, FARF Family Services Coordinator, at 1-541-687-4658 or registrymanager@fanconi.org. Suzanne will work with you to set up your profile and enter your information. Thank you for playing an important role in advancing treatments! ■

Cultivating Wellbeing in Our Lives



Megan Voss, DNP, RN

Do you feel content and balanced in your life? Do you live with a sense of purpose? Do you find ways to stay energized and connected to your community? Some people may answer yes to those questions, but for many, it's a struggle to manage stress, find purpose, and feel energized in our lives. With a disease like Fanconi anemia, most of our attention is focused on physical health. Mental health is a component that may get overlooked, yet it is crucial to our overall wellbeing.

Megan Voss, DNP, RN, is a nurse clinician, educator and program lead at the University of Minnesota Masonic Children's Hospital in the area of integrative health and healing. Megan joined us at Camp Sunshine this past summer to speak about wellbeing and why it's important to our quality of life.

What is wellbeing?

According to the model developed at the University of Minnesota, "wellbeing is the state of general contentment with life and the way things are." It has to do with empowering yourself to take charge of your health and life. "Wellbeing begins with the simple question—what can I do to feel content and balanced? Asking this shifts our whole perspective—we are no longer looking to our healthcare providers

or government or food companies to tell us what we need to do. We are empowering ourselves to explore what we really need and to evaluate for ourselves what makes sense."

As Megan explains, the healthcare system (drugs, hospitals, and doctors) affects only about 10% of the usual measures of health. The remaining 90% of health outcomes are determined by factors over which the medical system has little or no control, like lifestyle choices, social conditions, and our environment. That's why taking personal responsibility of our healthcare is so important.

So, where to start?

The model of wellbeing and integrative health created at the University of Minnesota includes

six dimensions of wellbeing: health, relationships, security, purpose, community and environment.

How to make a change?

If you're motivated to take charge of your wellbeing, a good place to start is by choosing one of the six areas where you want to make a behavior change. Visit www.takingcharge.csh.umn.edu to explore each component and to access dozens of resources. You can take a wellbeing assessment test which will help you identify patterns in this area of your life, set a goal, understand your strengths and challenges, and create an action plan.

Further resources:
www.takingcharge.csh.umn.edu ■

- Clean air and water
- Free of toxins
- Access to nature

- Livability (green space, culture)
- Equity (access to social, economic, political resources)
- Connectedness (engagement, empowerment)

- What gets you up in the morning?
- Aim
- Direction
- Different from a job



- Physical activity
- Diet & nutrition
- Sleep
- Thoughts & emotions
- Stress mastery

- Family
- Friends
- People in the community

- Basic human needs
- Job
- Finances



Robin and Jolandie

Expeditions

By Robin Lewis

I'd like to tell you about a journey that changed my life forever. As I imagine is the case for so many, I have had an interesting experience with Fanconi anemia so far. Although there are plenty of challenges that came with the diagnosis, I believe it has changed my life for the better. FA has taught me a couple of life lessons that you would expect to hear from a man late in his 90s, like: take everything in, and appreciate every smile, moment of laughter, and every single sunset. Even more, appreciate being able to get up in the morning and take a breath.

I have to be honest, there have been dark times my friends, very dark indeed. I think anyone who has experienced the uncontrollable feeling of actually hoping to die by the next morning would agree that FA is not all about beautiful, mind-altering moments. No, like everything else in life, sometimes it is just plain depressing. That said, we try to focus our horizons on the good stuff, and that's what makes it bearable.

My wife, Jolandie, and I had a pretty good thing going back in our hometown of Mbombela, South Africa. By age 25, I had successfully owned and operated two reputable businesses, had a lovely home, and plenty of people we called friends. We spent our weekends isolated in the bush, but worked extremely hard at chasing money and a sense of accomplishment during the week. We're a great team, and we spend every single hour of every day together.

Life was going well. Then, headaches and a racing heartbeat called for some concern. I went to get my blood pressure checked, and my life was never the same again. Government hospitals in South Africa are on another level (basement level, if you ask us) and we have plenty of stories to tell. I have had six bone marrow biopsies to date, four of which were done by students getting their practice in. It's not hard to believe that we struggled significantly with finding out what was wrong with me. Finally, I was referred to Groote Schuur Hospital in Cape Town. This saved my life!

I received a double diagnosis: myelodysplastic syndrome (pre-leukemia), and Fanconi anemia. What?! 'Yes sir, you will have to close up shop in Mbombela, and be in Cape Town for treatment by yesterday!' Our research into FA was frightening, to say the least, but explained quite a few things in terms of my life and body. My mother passed away in her 30s, as did her father. The diagnosis cleared up some of these questions.

We did what we needed to do, and I booked my bone marrow transplant (my sister was a 50% match). We spent a terrible five months in an isolation room. Graft-versus-host disease was my next competitor, and it almost won a couple of times. It's still in the fight, but we're beating it down one day at a time. When I was discharged from the hospital, I could not walk, and I was wearing adult diapers. I did not have the strength to unlock a door, and I was getting fed through a tube in my arm. God blessed me with a wife who pulled me up from the pits of darkness. She bathed me in bed, slept on the floor next to me, and gave up the chance of one day becoming a mother. Wait, as I write this, I'm tearing up. Let me take this to the good side of things.

Numinous Expeditions

Let's take it back to 2017. Jolandie and I had this dream of one day retiring and driving across the world. We also enjoyed helping others in our daily lives. It just felt like we were destined to do so. One evening I was sitting on a hospital bed, and the doctors were looking very worried about what they were seeing in my blood tests. They had not given me any diagnosis yet. I was alone, and I had just helped another patient with a minor problem.

continued on page 12



Did you know there's a podcast about Fanconi anemia?

This is thanks to Daniel Kold, an adult with FA from Denmark. In the podcast, entitled "Life, Death, and Happiness", Daniel interviews those living with a chronic or terminal illness about those three topics. We asked Daniel to tell us more about the project, how people can listen, and what his plans are next.

What inspired you to start a podcast?

I have wanted to create awareness about Fanconi anemia for a long time. I used to raise funds, but since it is illegal to fundraise for non-Danish organizations in Denmark, I decided to switch my focus and help another way.

I have been running a different podcast with three friends for almost a year and a half. It's a fun project in which we talk all about gaming. Through this project, I learned how to host and edit a podcast. One day, it occurred to me that I wanted to do a different kind of podcast – one that would actually make a difference. From there I worked to come up with a concept. It took me a while, and I actually talked to some FA friends about it early on.

Why "Life, Death, and Happiness"?



The most important aspect for me was that I did not want to make something about how terrible life can be. I wanted to approach these topics from a different angle. For me, it's so important to find the positive in the negative.

Hopefully this podcast will create some awareness for "normal" people about what it means to live with a chronic disorder or a terminal illness. It's never fun to deal with these things, but on the other hand, it doesn't have to feel like the end of the world. My hope is that people going through hard times are uplifted and maybe even inspired to go out there and live a full life. The way I look at it is this: yes, my body doesn't work like it's supposed to, but that doesn't mean I can't live out most of my dreams, get the job I always wanted, or maybe even start a family.

How can people listen?

People can listen for free through most podcast apps and programs. You can search for the name "Life, Death & Happiness" on most major platforms like Apple Podcast, Spotify, Overcast, Anchor, Breaker, Castbox, Google Podcast etc. Most of these platforms have free apps on both Android and iPhones and you can also find several of them on their websites, so you can access all episodes from your computer, too.

If you subscribe (this is also free) to the podcast on any of these platforms, then you will get a notification every time a new episode goes live.



What are your plans going forward?

I would like to continue to expand the podcast by interviewing new guests who have FA or other chronic diseases. If I can afford the right equipment, I would also love to go to the FA Adult Meeting, Camp Sunshine, and other places to make special episodes. My dream is to make episodes where a small cast of up to four people can sit down face to face and talk about specific topics like: How do you deal with parenting? How can you best prepare for a bone marrow transplant? How did your family react to your FA or cancer diagnosis?

Are there specific moments from the podcasts that you'd like to share?

In episode five, I talked to Allison, the wife of Sean, who has FA. I said to her, "Sean has been through so many cancers. It's so terrible. But at the same time, I think – man, what a fighter". Her answer was just so brilliant: "Ah yeah. We talk about this all the time, and we don't love these terms. You know – fighting, and battle. But we haven't found a good replacement. I think there's a reason he doesn't love that. He feels like what if he loses? People could say he lost his battle with cancer. And it sort of feels like it's your own fault right? And it's not."

continued on page 12

Expedition, continued from page 10

Something came over me and I closed my eyes and proceeded to make God a promise. I said: "God, if you would give me 10 more years, I'll make it count. I'll do what is right: fight for those who can't, protect the things that need protection, open my heart to others, and help where I'm needed." I vowed not to chase money or success, but to focus on the things that really matter. A while after that, I got diagnosed with FA. They told me that if I were to survive the transplant, my life expectancy would be 35. I was 25 on the evening that I asked for my 10 years. Enough said. Numinous Expeditions was born.

We jumped into action and built a camper on the back of my work truck. We rushed to get it done before treatment started, worked straight through the last night, and left for Cape Town for treatment the next day. The plan was to drive around South Africa and help anything and everything that we agreed needed the help.

We did our first major animal project two weeks after I was discharged from hospital. We drove 2000 kilometers assisting animal shelters and sanctuaries in any way they needed. We decided that we wanted to make a bigger impact, and the idea of doing a global expedition grew quickly. We managed to purchase an old Mercedes truck that we are currently busy re-building into a home on wheels for our global trip starting with the African continent. Our goal is to do one major humanitarian, environmental, or animal welfare project in every country we reach.

We don't necessarily look for projects. They just seem to find us. We motivate other bone marrow transplant

patients to win the fight in the isolation ward in Groote Schuur hospital, and have just started working on our next project. Five individual artists will be filling the isolation rooms with color and creativity, and hopefully it will make a difference in the mental state of patients booked into the ward. We plan on funding our living costs from hand-crafted jewelry that we make from our home on wheels.

I will do everything in my power to succeed in making a lasting impact wherever I go and better the lives and wellbeing of others. I aspire to inspire other FA warriors to fight the good fight, and succeed in whatever they put their minds to, because nothing is impossible! ■

Find Numinous Expeditions (NUMEX) on Facebook or at their website www.numinousexpeditions.co.za.



Robin and Jolandie with Betsy, their house on wheels

Podcast, continued from page 11

This spoke to me and gave me a lot to think about. Even if we mean well, calling each other warriors and fighters like that, it also indicates that this is a fight we have chosen. And it's far from that.

Another highlight for me was when I asked Kyle (an adult with FA) in episode six about some good advice to young people diagnosed with FA or cancer. What would he say to someone who is trying to deal with both being young and also having a disease? Part of his answer was this: "*The only thing that is certain is uncertainty. So many things that are against the odds have been happening to me. So many things have happened to me that*

usually wouldn't happen to any normal person. I've learned that uncertainty is what really gives our life flavor. It's not knowing what is going to happen next, but also kind of being excited about it."

This way of looking at the challenges we face is something I've thought about a lot. I think I can honestly say that I've walked away from each episode with new knowledge and often also with a changed perspective on life, death & happiness. I love to give people the chance to tell their story, and I also enjoy learning about so many wonderful ways of dealing with life and its challenges. ■





2019 Family Meeting in Photos

This year, more than 50 families gathered at Camp Sunshine in Maine for the annual Family Meeting. Here are some of the highlights. To learn more about this meeting and how to apply for 2020, visit our website www.fanconi.org.



Remembering loved ones at the balloon release



Celebrating fundraising success



Getting together with other adults with FA



Recognizing the important work of Ralf Dietrich



Letting loose with some karaoke



Learning from the FAdult Council

WHY RESEARCH MATTERS

How our family channeled grief into hope

By Zachary and Rachel Gratz-Lazarus

Our Mighty Mouse

Well before her diagnosis of Fanconi anemia (FA), our daughter Norah was a model in resiliency for us. Born at just five pounds and three ounces, she had the strongest heartbeat on the hospital floor, surprising us and her doctors. It was a high-risk pregnancy because she wasn't growing as much as she should have been. In the third trimester, we had to attend non-stress tests several times a week to make sure that Norah was not under any distress. Norah's mom was scheduled for an induction, much to our chagrin.

Despite all of the doctor's concerns, Norah was a rockstar. We ended up having an eight-day induction. Though Norah was not quite ready to join us, she showed no signs of ill health. The head OB/GYN even sent us home for 24 hours halfway through the induction.

When she was finally born, we called her Mighty Mouse. Small, but resolute. Our model of resilience. Our first born. Our Norah.

The Diagnosis

We received Norah's diagnosis in stages. She was born with hearing loss that turned out to be progressive

in at least one ear. Her doctor was concerned. Progressive hearing loss in children is rare. Progressive loss in one ear, even more so. The pediatrician sent us to get a blood draw (the first of many) and referred us to the genetics department. When the blood tests returned with decreased platelets, we knew something wasn't right.

We first heard the words "Fanconi anemia" over the phone with our pediatric hematologist/oncologist. Getting the diagnosis sent us into a tailspin. We cried every day for weeks. Norah's dad immediately regretted a decision he made in college to not take as many science classes. A DNA-repair disorder? We had our work cut out for us.

Bewildered, we started doing our homework. What's the course of treatment? Who in our communities has expertise in FA? Most importantly, what can we do to take control over our circumstances and best care for Norah?

And so, with the FA diagnosis in hand, our family started putting the puzzle pieces together. We learned that hearing loss and small stature were related to Norah's diagnosis, among other challenges. We reached out to the Fanconi Anemia Research Fund

(FARF), read what we could stomach and watched videos on cell biology so we had a better sense of what the doctors were discussing. We turned to Norah, our dancer, singer, and bookworm, for help staying present.

Lucky is a challenging word to describe our change of circumstances, but despite our ignorance in cell biology, we felt lucky to have some of the tools we needed to face this challenge head on. Rachel works in public health doing policy work and has a background in early childhood education, race and health equity issues. Zach has worked in community organizing and as a social worker in community mental health settings. We started taking the bull by the horns: channeling our grief into hope, teaching our children to love themselves, and fighting for life.

The Norah Needs You Campaign

Part of the FA diagnosis is facing the reality that Norah may one day need a bone marrow transplant. When we learned that there were no perfect matches in the bone marrow registries, we reached out to Be the Match and Gift of Life (which specializes in working with Ashkenazi Jewish populations) to build bone marrow donor campaigns. We leaned into our community to host bone marrow drives, connect us with media, and to help publicize the search. Rachel used her incredible skills as an artist to build a website (www.norahneedsyou.com), while Zach reached out to an old friend in the California State Assembly. With the help of State Assemblyman Marc Berman, we passed legislation to encourage people to join the bone marrow donor registry. The work

snowballed from there. As of July 2019, we have registered roughly 8,000 people as a part of our Norah Needs You campaign.

None of this, of course, would have been possible without the tremendous support from our friends, family, co-workers and community at-large. And it has not come without hesitation: we are constantly concerned with the emotional well-being of both of our children (including our three-year-old son Ellis, who does not have FA). Being so public with FA feels scary. And, while being the parents of a child with FA is our story, actually having FA is not. We have robbed Norah, to some degree, of her anonymity in the hopes of finding her a bone marrow match and to help save as many lives as we can. We hope that, with time, this knowledge gives her pride and a sense of meaning.

Gene Therapy

Within months after Norah's diagnosis, we learned about a gene therapy trial that had just opened 25 miles from our house. Our timing was serendipitous. After a series of referrals, we reached Stanford's gene therapy team just a couple of months shy of the opening date of the trial. Despite the exploratory nature of clinical trials, the team had data from two prior studies that demonstrated both a lack of negative short-term effects and hope for the possibility of avoiding a transplant.

Blessed that the trial was opening, that Norah was eligible, and that there was a spot available, we did our best to make an informed decision. Within months of the diagnosis, we had to make a decision whether to enroll our daughter in this experimental trial. We



Norah with her parents (top), at the California State Assembly (bottom left), and with Dr. Agnieszka Czechowicz at Stanford University for the gene therapy trial (bottom right).

needed to talk with the experts. We spoke with doctors at Fred Hutch in Seattle, Cincinnati Children's Hospital, University of Minnesota, Children's Hospital of Philadelphia, University of California San Francisco, Dana Farber Cancer Center, and others. Through FARE, we reached out to parents whose children had participated in previous gene therapy clinical trials.

The information we received was

overwhelming and confusing: risks unknown, benefits questionable. But, at the end of the day, the level of monitoring was going to be similar whether we went forward with gene therapy or not. Either way, regular blood draws and biopsies were in our future. The question was: would we have hope for improvements? Or simply hope for stability and a slow,

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FANCONI ANEMIA GLOBAL NETWORK

You probably already know that FARF is an international organization, but did you know that there are several partner FA organizations around the world? Some are run by FA families, some by doctors and some by FA researchers.

Last year, more than 40 researchers, clinicians, FA family members, and representatives from FA organizations around the world got together to build a more intentional global FA network. Participants addressed organizational and fundraising capacity, family services, and access to doctors and medicine. Together, the group determined needs, priorities, and action steps in each of these areas. We are excited to work with our international partners to develop a more cohesive network of FA research and support services.

Spain

Fundación Anemia de Fanconi

<http://anemiadefanconi.org>
Aurora de la Cal Villamayor
aurora.delacal@ciemat.es

Portugal

Associação Portuguesa Para A investigação da Anemia de Fanconi

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International Support Grants Awarded

Although the FA Research Fund is based in the United States, FA research, families, and support organizations span the entire globe. To strengthen the efforts of our partners in the international FA community, FARF established the International FA Support Grant program. This past spring, FARF awarded three \$10,000 grants for one-year projects that address priorities determined at the 2018 FA International Summit. These priorities were: access to medicine, doctors, and facilities; organizational and infrastructure needs and access to family support services.

2019 Grant Recipients



Group: Fanconi Hope & Fanconi Anemie Werkgroep of the VOKK (Dutch Association of Parents with Children with Cancer) Nederland

Countries: United Kingdom and Netherlands

Project: Creation of an FA Europe Group. This project aims to unite the existing FA support groups across the 23 member states of the European Union into an umbrella group that will increase collaboration between countries, as well as augment partnerships with EU healthcare networks and pharmaceutical companies. FA Europe will connect patients, clinicians, and scientists, and act as a facilitator for fundraising, sharing best practices and collaborating on research efforts. The group will use the grant to carry out

the start-up meeting between countries in April 2020. This project is led by the Fanconi Anemie Werkgroep in the Netherlands and Fanconi Hope in the United Kingdom.

Amount awarded: \$10,000



Group: Un Corazon por Fanconi

Country: Dominican Republic

Project: Diagnostic protocol and support for FA patients in the DR. Un Corazon por Fanconi is a newly established foundation currently focused on raising awareness and funds. This project focuses on developing a diagnostic protocol for FA patients in the Dominican Republic, providing support services for FA families, and building organizational capacity. With help from experienced FA researchers in Mexico, the team in the DR will work to form a network of doctors who can learn about the diagnosis and management of FA. This project is led by an adult with FA, Ana Tabar.

Amount awarded: \$10,000

Group: Fanconi Anaemia Support Australasia (FASA)

Country: Australia and New Zealand

Project: Creation of an FA support group in Australasia. The aim of this project is to establish a support group for families and individuals with FA in Australasia, which will help build knowledge of FA within the existing medical infrastructure. A relatively small population of people with FA spread out around Australia and New Zealand results in a lack of experienced FA specialists. Fanconi Anaemia Support Australasia will formalize efforts started by St. Vincent's Institute in Melbourne to engage with the local FA community, build awareness, and build a stronger medical infrastructure. This project is led by an FA family (the Hawkshaw family), with support from FA researchers.

Amount awarded: \$10,000



Amelia Hawkshaw



Wayne Crismani, PhD

The 2020 Grant Cycle

We will begin accepting grant proposals in spring 2020. Look out for email updates early next year. ■

A Pursuit in Genetics



By Anne Starner

In my early high school days, I noticed that I had a knack for biology, and specifically, genetics.

It has always made sense to me and it sparks joy in my life. I know what you're thinking – very nerdy. This joy for biology continued into my college career. Originally, I was going to apply my love of science in the Air Force. I wanted to work in their chemistry or engineering departments to help create military technology. As time went on, my goals changed and I became more interested in genetic research. Unfortunately, the Air Force did not have a department for this, so I decided to pursue flying drones for the military. However, this just didn't capture my interest like genetics did.

During this period of weighing my interests and possible career paths in the Air Force, I started to have some unexplainable medical problems. I was rushed back home to Florida in order to get a diagnosis at Moffitt Cancer Center. After a surgery to fix my broken talis bone (a small bone in your ankle), and many visits to the hematologist, I was finally diagnosed with Fanconi anemia at the age of 20. Soon after, my family and I found out that my brother was a perfect bone marrow match for me. We actually received this call while we were lying on the beach – such a

beautiful background for one of the most important phone calls of my life. I was raced to transplant shortly after being diagnosed and had a textbook transplant on October 12, 2016.

Since then, I have gone back to college to finish my bachelor's in biology that I started in the Air Force. Due to the fact that I have FA, the Air Force would not allow me to go back. As much as I fought them on my discharge, I believe that it was a blessing in disguise. I finally have a platform that will allow me to work in genetics.

This summer, I had the honor of working in Dr. Alan D'Andrea's lab at the Dana-Farber Cancer Institute in Boston. After meeting Dr. D'Andrea at the 2018 FA Adult Meeting and Symposium, I knew that I had to learn from him. He quickly agreed to having me work in his lab for three months, where I learned from some of the most amazing researchers. As the nerd that I am, I found the smallest task and machine to be exciting. I assisted in some drug testing, looked at how drugs affected certain proteins, and many other experiments for two of the post-

docs working on improving the lives of people with FA. These researchers come from around the world and usually do not have any personal investment in FA. However, they are so dedicated to their work. Most weekends, you will find them at the lab doing experiments. They showed me the dedication and skill that it takes to be a researcher. They always believed in me, even after a big mistake. The people who research our disease are just as amazing as the people who have the disease.

After coming home and getting ready for my final year of college, I have to believe that I left an impression on the researchers there. I hope that I left them with a sense of how much people with FA fight and count on their work. I also hope to have inspired them with my story to stay focused and dedicated to finding answers about this disease. There is no doubt in my mind that these researchers can discover new treatments to give us longer lives with a higher quality of living.

As I look towards the future, I have my eyes set on graduate school to get a PhD in genetics. I plan to use this degree to research FA and maybe even find a cure. This fall I will be completing applications for graduate programs and going to interviews come winter and spring time. I do not know where this journey will lead or which state it will take me to, but I am excited for the adventure of a lifetime. I just hope that this typical Florida girl can find some place warm to study for the next few years ☺ ■

“ The people who research our disease are just as amazing as the people who have the disease. ”

In Loving Memory

Ryan
Hinshelwood
3.14.85 – 5.26.19

Danielle
Folkes-Blake
2.26.84 – 4.27.19

Anne-Marie
Malmgren
1.5.71 – 3.17.19

Sanjeev Parmar
12.16.79 – 9.20.19

Ruthie Saunders
4.12.81 – 7.18.19

Lilou Klug
11.1.18 – 9.16.19

FAMILY FUNDRAISING EFFORTS

From January through August 2019, FA families raised more than \$1,000,000 for the Fanconi Anemia Research Fund! 183 families raised funds, with 97 raising at least \$500. Each dollar donated advances research and family support, making a difference for all those affected by FA and their families. Sincere thanks to every family and individual who worked so hard to raise funds in honor or memory of loved ones.

\$200,000+

The Kendall and Taylor
Atkinson Foundation with
the Nash and Griggs Families

\$100,000 - \$199,999

Lynn Frohmayer
Orion and Lisa Marx

\$50,000 - \$99,999

Kevin and Lorraine McQueen
Gerard and Cynthia
Vandermeys

\$25,000 - \$49,999

John and Kim Connelly
Tim and Mary Ann Lana
Todd and Kristin Levine

\$15,000 - \$24,999

Carly and Brian Adel
Mauro and Kerrie Cazzari
John and Martina Hartmann

\$10,000 - \$14,999

Rachel Altmann and Tyler
Morrison
Stephen and Jennifer
Klimkiewicz
Neil and Emily Robison

\$5,000 - \$9,999

David and Sarah Borden
David and Mary Ann Fiaschetti
Erin Furr
Susan and Skip Longstaff
Kevin Gatzlaff and Rachael
Alaniz
Brian Anderson and Sultana
Graham
Rachel and Zachary Gratz-
Lazarus
Charles and Kathleen Hull

Ian and Tricia Mitchell
Peg Padden
Peter and Janice Pless
Paul and Rena Rice
Nigel and Ann Walker

\$1,000- \$4,999

Michael and Jennifer Aggabao
James and Jennifer Armentrout
Amanda Barber
Mark and Linda Baumiller
Jennifer Bland
Chris and Jennifer Branov
Ryan and Rebecca Brinkmann
David and Kim Chew
Andrew Coons and Valeen
Gonzales
James and Crystal Eubank
Justin and Britteny Ferrin
Andrew and Jennifer Gough
William Graham
Alan and Rachel Grossman
Owen Hall and Margaret
Kasting
Andre Hessels and Rutger
Boerema
Stan and Michelle Kalemba
Dan Klug and Elizabeth
Bertrandt-Klug
Robert and Anna Langtry
Brian Horrigan and Amy Levine
Keith and Jessica Loo
Sheila Meehan
Robert and Mary Nori
Ronald and Fredi Norris
Nancy Nunes
David and Stacy Ownby
The Pearl Family
Janice and Kenneth Sysak
George and Kathy Reardon
Mark Ritchie and Lisa Mingo
Craig and Alisha Rushing
Andrea and Bob Sacks
Sharon Saunders

Bryan and Karen Siebenthal
Ana Alejandra Tabar Concha
and Elvin Estevez Lopez
Devon and Bob Tessier
Bill and Mary Underriner
Jessica and Ezekiel Werden
Bob and Julie Williams
Cecelia Zurhellen

Up to \$999

Peter and Donna Abramov
Victor and Mary Albino
Charles Balow and Xandra
Towndrow
Israel and Mary Jo Becerra
Adam and Marissa Becker
Jasmine Bennetsen
Domenico Bertolucci and
Frederica Bonati
John and Elanie Beyer
Tracy Biby
Jeff and Donna Boggs
Richard and Tena Boson
Sean and Allison Breininger
Donald and Danielle Burkin
Liz and Richard Butts
Thomas Bynam
Robert and Barbara Capone
David Guidara
Stuart Cohen and Deane
Marchbein
Paula Corredor Lopez and
Matthew Garisch
Mark and Annelie Crean
Lea Ann and Jeff Stiller
Larry Davis
Darrel and Kalani DeHaan
Jeremy and Michelle DellaValle
Egil Dennerline and Nanna
Storm
Robert and Dawn Desmond
Marie Di Mercurio
Scottie and Jessica Dill
Cleone DiSandro

Sharon Swanson
Chloe Eminger
Billy Joe and Debra Estep
Daryn and Carol Franzen
Fabio and Sune Frontani
Melody Ganz
Mitzi Gerber
Gary and Heidi Grassi
Eugenio Grassi and Brittany Miller
Michael Greenberg
Madeline and Patrick Gregg
Ben and Stephanie Griggs
Eric and Elisabeth Haroldsen
Bob and Victoria Hathcock
Patricia and Michael Hilbert
Estate of Robert Hutchins
Shane and Colleen Irvin
Jeff and Beth Janock
Nancy Jansen
John and Karilyn Kelson
Christopher and Dana Lamb
Eugene and Renee Lemmon
Peg LeRoux
Tanner and Jessica Lindsay
Col Gregory & Lt Col Lynnette Lowrimore
William and Jacquelyn Lucarell
Kristina Mack

Linda McCarthy
Peggy McDaniel
Catherine McKeon
Daniel and Angie McMahon
Johan and Emel Mehlum
John and Barbara Miller
Kelly Mlachak
Griff and Cecelia Morgan
John and Betty Mozisek
Kenny and Lisa Myhan
Tony and Lina Nahas
Lisa and Jack Nash
Jack and Tammy Neal
Alice Nicholson
Anne Park
Leah Petsanas
Marcos and Silvana Pineschi Teixeira
John and Dianne Ploetz
Ashley Power
Mike and Kay Proctor
Lynn and Shirley Quilici
Pedro and Marina Ravelo
Kelsey Robinson
Kevin and Katherine Rogers
Les and Nancy Ross
Stanley and Lisa Routh
Richard and Marilyn Sablosky

Jennifer and Brian Sadlowe
Mayra Lemus
Richard and Dolores Satterlee
William and Marisela Schaecher
William and Connie Schenone
Colleen Scholl
Jim Siniawski
Karin Staab
Adam and Jennifer Stewart
Bruce, Loreen, and Jack Timperley
Mark and Susan Trager
Tom and Cathy Uno
Mike and Beth Vangel
Cristal Vigil
Theresa and Louis Viola
Joseph and Natalie Vitrano
Ira and Terry Walker
Marc Weiner
Michael and Kimberly Williams
Alexander Winn
David and Marivel Winn
Werner and Laetitia Wolfswinkel
Chad and Dawn Wood
Kyle and Madison Wright
Jian Yang and Jing Nie
Sean and Kristin Young

A note to our fundraisers: we greatly appreciate your efforts to raise money for FARE, and we want to recognize you all accordingly and with 100% accuracy. If we have inadvertently made an error, please let us know by emailing info@fanconi.org. Thank you. ■

DONATE WHILE YOU SHOP ON AMAZON

AmazonSmile donates 0.5% of the purchase price of eligible products to selected charities. Visit smile.amazon.com, select the Fanconi Anemia Research Fund as your charity, and start shopping!



FIND ANSWERS

FIGHT ALWAYS

MAY 2020

#THISISHOWIFA

Get ready for the biggest FA Day yet!



Hello, I'm Julia!

Are you curious about fundraising? Excited? Intimidated? Not sure where to start? Or, are you ready to take your fundraising to the next level? I'm here for you!

My name is Julia Wong and I joined FARF earlier this summer as Family Fundraising Program Manager. This is a new position completely dedicated to YOU, individuals with FA and families who are interested in raising money for research and support programs. It's incredible to look at the impact you all have made on FA research over the last three decades. What this small community has done to change outcomes for individuals with FA is remarkable!

We all know we still have a long way to go, but now we know just how much of a difference our fundraising makes. I'm here to help make the impact of fundraising even greater. I do that by working individually with anyone interested in raising money. I can help you with any or all of the following:

- Choosing the right fundraiser for you
- Setting a goal
- Getting logistics straight for your event or appeal
- Setting up a fundraising page for you on FARF's website
- Printing & mailing invites or letters as needed
- Navigating sponsorships
- Sending swag and materials for your event
- Helping you thank your donors & show them the impact of their gifts
- **Being a cheerleader & sounding board for you throughout your fundraising efforts**

Call me at 541-687-4658, email me at julia@fanconi.org, or find me on Facebook and we'll start a conversation. I can't wait to work with you! ■

HOW TO MAKE A DIFFERENCE THIS HOLIDAY SEASON

Did you know that 70% of FARF's income is generated by holiday gifts? Many of these are the result of families like yours asking your communities to make a gift in honor of your family. The median sized gift made during the holidays is \$60. If 10 of your friends or family gave that much, you would raise \$600 for research and support services. We know you're busy, especially during the holidays, which is why we've made it super easy for you to invite your community to give.

YEAR-END FUNDRAISING CHECK-LIST:

- DECIDE:** do you want to send a traditional printed letter, an email, or have a customized donate page on FARF's website? To increase your impact, do all three!
- CONNECT:** if you're sending a letter, email Julia at julia@fanconi.org and she will help you format your letter & photos, then work with you to get your mailing list, print, stuff & send your letters – no need to worry about postage!
- CREATE:** if you're going the online route, all you need to do is visit <http://fundraise.fanconi.org/2019holidays> and click "become a fundraiser". Add your photo and a personal message to your friends. We've already added the impact of fundraising dollars for your donors to see. All you need to do is then share your page with your community.
- CELEBRATE!** You've made a difference! We will connect with you in early 2020 with a fundraising report so you know the impact you and your community made.

Sample print letter to send to your friends & family this holiday season:



Dear friends,

As we reflect upon 2019, we are incredibly grateful for a year full of cherished memories with our family and friends. Having a child/ren with a rare disease is a constant reminder of what is important in life. For us, what's important is _____.

[Update on your family this past year – trips? School milestones? Health updates? Etc.]

As you know, our son/daughter/child/children _____ lives with Fanconi anemia, a rare DNA-repair disease that leads to bone marrow failure, leukemia, and cancer. There's currently no cure, but there is very promising research. That is where your support means so much. The Fanconi Anemia Research Fund (FARF) was started in 1989 to find effective treatments and a cure for FA and to provide education and support services to affected families worldwide. FA research cannot move forward without funding. Great advances in understanding this disease have been made, but more needs to be done to find a cure.

The survival of people with Fanconi anemia is in our hands. You can be part of the breakthrough. When you give to the Fanconi Anemia Research Fund,

- You're funding clinical trials to advance therapies for kids and adults with FA and you're supporting research in gene therapy, gene editing, and advances in bone marrow transplants.
- You're investing in better outcomes for everyone touched by cancer. Both Fanconi anemia and cancer are the result of the inability to repair DNA. By funding FA research, you're helping to unlock the mysteries of DNA repair problems that will impact not only cancer treatments for FA patients, but also cancer treatments for everyone else.
- You're providing individuals with FA and their families with services we rely on, like helping to navigate the FA diagnosis, in-person support meetings, research opportunities, educational materials, and opportunities to network with other FA families around the world.

We ask for your support to find a cure for _____ and others with FA. You can send your tax-deductible gift back in this envelope or give online at www.fanconi.org/donate.

Thank you so much for your love and support.

Wishing your family a wonderful holiday season!

The ____ Family



Sample email to send to your friends & family this holiday season:

Dear _____,

I'm writing to let you know this holiday season I'm fundraising for the Fanconi Anemia Research Fund (FARF). As you know, our son/daughter(s), _____, has Fanconi anemia, so this organization is very close to our hearts.

FARF funds research for better treatments and a cure for Fanconi anemia. They also provide support services to affected families like ours. I'll be asking pretty much everyone I know to make a donation, but I thought I'd ask my closest family and friends first. Will you help me reach my goal of _____?

You can check out my fundraising page by clicking this link: _____.

Any support you can give means so much to us. Thanks for reading and helping me support this important cause.

Thank you and happy holidays!



FUNDRAISING SCRAPBOOK



Three Sisters Challenge

This past August, board member Sharon Schuman took on three physically demanding challenges in memory of the Frohnmayer sisters, Kirsten, Katie, and Amy. Sharon completed a 100-kilometer bike race, ran a half-marathon in the Oregon rain, and was accompanied by Lynn Frohnmayer and Mirjana Covington for a hike up the 10,000 foot South Sister Mountain in Oregon. The Three Sisters challenge raised over \$5,000! You continue to impress, Sharon!



Your Rope Team Climb

Friend and board member Bill McCoy is no stranger to incredible physical feats. This summer, Bill challenged himself to two difficult climbs in honor of those with FA. In June, Bill and the Rope Team successfully summited Mt. Fuji in Japan. On their second adventure, Bill and his team climbed up to 8,000 feet on the Sahale Mountain in Washington before making the tough decision to climb down due to extreme weather conditions. The Rope Team raised over \$40,000 for FA Research. Thank you, Bill & Your Rope Team!



Evan's Enchanted Evening and Becca Too!

The Connelly family hosted their 10th Evan's Enchanted Evening and Becca Too fundraiser, which raised over \$60,000! The event featured great food and drinks, a silent auction, and an inspiring address from FAdult council member Jack Timperley. Every other year, the community of De Pere, Wisconsin, comes together to celebrate the Connelly family and support all those living with FA. Thank you, Connelly family & friends!



Lanapalooza

This past June, after hosting their 5K for FA and raising \$25,000, Mary Ann, Tim, and Eli Lana stepped up for another fundraiser, this one in memory of Eli's uncle, Joseph Lana. The communities of Rochester and Hilton, NY, came together to remember Joe, a celebrated local musician. Lanapalooza raised over \$14,000, and both Tim and Eli got on stage to play the drums – a true tribute to Joe. Thank you, Lana family and all those involved with Lanapalooza.



Team Bravery

Just when you think they can't get any more impressive (they ran 100 miles last year!), Team Bravery does it again! Orion Marx, Charlie Scott, and Zar Toolan ran 109 miles along the Mickelson Trail with the hope of raising \$1,000 per mile. Not only did they meet their goal, but they exceeded it, raising a total of \$118,000! Thank you for your incredible efforts, Team Bravery, and for inspiring all of us to take on big challenges, turn to our communities for support, and do something epic.



The Werden Family

Inspired to make a difference, Jessica Werden of Indiana jumped into fundraising this past spring. She set up an FA Day fundraising page in honor of her two young kids with FA, Sloane and Remington. By the end of FA month, the Werden family and friends donated over \$1,800! Jessica was encouraged and partnered with a local bookstore to hold a fundraising event during the summer. We want to thank the Werden family and Books and Brews in Noblesville, Indiana, for raising another \$1,800. Incredible!



Nina's Starry Night

This year, Rachel Altmann and Tyler Morrison hosted the 6th annual Nina's Starry Night in memory of their daughter Nina. This colorful and festive event included a Bollywood dance party, silent auction, and an incredible speech by FAdult council member, Jasmine Bennetsen. This year, Nina's Starry Night raised over \$8,000! Thank you, Rachel and Tyler!



Becker Family Wedding Donation

Valerie Mauriello and Nicholas Scofield, family friends of Adam and Marissa Becker – whose son Zach has FA – tied the knot this September. In lieu of wedding favors, Valerie and Nicholas made a gift to FARF in honor of Zach. Thank you for thinking of us on your special day – talk about good karma to kick off your marriage!

From Nov. 1, 2018 – Sept. 30, 2019, your contributions allowed FARF to award **\$1,555,424** to the following projects:

YOUR FA RESEARCH DOLLARS AT WORK

HEAD AND NECK CANCER

Project: Gefitinib/Afatinib therapy for Head and Neck Squamous Cell Carcinomas in Fanconi anemia

Issue: Beyond surgery there is currently no effective therapy to treat people with FA who develop solid tumors.

How will this project address this issue?

Researchers will examine two currently FDA-approved drugs as potential treatments for solid tumors in FA. This work will lay the foundation for an anticipated clinical trial in 2020.

Donor contribution: \$100,000

People behind the project: Jordi Minguillón, PhD; Jordi Surrallés, PhD

Institute: Institut de Recerca de l'Hospital de la Santa Creu i Sant Pau, Spain



Jordi Minguillón, PhD



Jordi Surrallés, PhD

Project: Squamous Cell Carcinoma Chemoprevention Phase 1

Issue: Head and neck cancer is a major issue for the FA population. There is no suitable preclinical model to study potential strategies for preventing or delaying squamous cell carcinoma in FA.

How will this project address this issue? Using a cancer-prone mouse model developed by a lab in the United Kingdom, researchers will test small molecules that could prevent or delay tumors in these mice. Results will then be used to inform and guide prevention trials in humans.

Donor contribution: \$27,700

Person behind the project: Markus Grompe, MD

Institute: Oregon Health & Science University



Markus Grompe, MD

Project: High-priority agents for Fanconi anemia-associated oral cancer treatment and prevention

Issue: As people with FA are living longer, the occurrence of oral cancer is increasing. These cancers are aggressive and require innovative treatment and prevention measures.



Frank G. Ondrey, MD, PhD, FACS

How will this project address this issue? Certain diabetes drugs have shown anti-cancer activity in the general population. Researchers are investigating if these drugs can reduce the growth of oral cancer in people with FA.

Donor contribution: \$237,457

Person behind the project: Frank G. Ondrey, MD, PhD, FACS

Institute: University of Minnesota

Project: Treating Fanconi Anemia Cancer with Proton Precision Therapy

Issue: Cancer in the general population is often treated with surgery and toxic conditioning measures, like chemotherapy and radiation, that are especially harmful to people with FA and can damage surrounding tissue.

How will this project address this issue? Proton-based radiation therapies precisely target the tumor and cause limited damage to the surrounding tissue. Researchers will examine sensitivity, toxicity, and biological response in cancer cells that mimic those found in people with FA to evaluate the potential of proton therapy in FA.

Donor contribution: \$200,000

People behind the project: Yi Zheng, PhD; Mathieu Sertorio, PhD

Institute: Cincinnati Children's Hospital Medical Center



Yi Zheng, PhD



Mathieu Sertorio, PhD

Project: Complete DNA sequencing of the Fanconi anemia patient tumors

Issue: Squamous cell carcinomas that people with FA develop are not well understood at a basic science level, which is needed in order to know how to treat and prevent them.

How will this project address this issue?

Researchers are using DNA sequencing technology to identify mutations that have contributed to the development of these cancers. Understanding these abnormalities allows the team to identify potential targets for treatment.

Donor contribution: \$90,862

Person behind the project: Agata Smogorzewska, MD, PhD

Institute: Rockefeller University



Agata Smogorzewska, MD, PhD

Project: Synthetic Lethal Approaches to Treatment of FA Gene Mutant Head and Neck Cancer

Issue: Typical treatments for cancer involve conditioning that damages DNA and can therefore be harmful for people with FA.

How will this project address this issue?

Researchers are testing drugs using patient-derived xenografts (tumors from patients that are grown in mice) to find a way to kill cancer cells without harming normal cells.

Donor contribution: \$157,500

People behind the project: Gary Kupfer, MD; Barbara Burtness, MD

Institute: Yale University



Gary Kupfer, MD



Barbara Burtness, MD

METABOLISM

Project: Metabolic alterations in glucose utilization and carnitine biosynthesis impact nutritional status in individuals with FA

Issue: Many people with FA voice concern about being underweight and unable to build muscle mass. This impacts not only the physical health of individuals with FA, but also their emotional and mental wellbeing.

How will this project address this issue?

Researchers are looking closely at the metabolism of people with FA to understand why they have a low body mass index.

Donor contribution: \$292,405

People behind the project: Lindsey Romick-Rosendale, PhD; Adam Nelson, MBBS, FRACP

Institute: Cincinnati Children’s Hospital Medical Center



Lindsey Romick-Rosendale, PhD



Adam Nelson, MBBS, FRACP

LEUKEMIA

Project: Modeling hematopoietic clonal evolution in Fanconi anemia

Issue: People with FA are at increased risk of developing leukemia at a young age and leukemia in FA is difficult to treat. Our understanding of the specific genetic mutations that occur in human FA blood cells to drive leukemia is poor due to the lack of disease models.

How will this project address this issue?

Researchers have developed a system to generate human FA blood stem cells which they will use to determine the key genetic mutations that drive the development of



George Q. Daley, MD, PhD



R. Grant Rowe, MD, PhD

leukemia. Identifying these mutations may lead to improvement in screening for and treating blood cancers in FA.

Donor contribution: \$200,000

People behind the project: George Q. Daley, MD, PhD; R. Grant Rowe, MD, PhD

Institute: Boston Children’s Hospital

GENE EDITING

Project: CRISPR transcriptional screens for re-activation of HDR in FA patient-derived cells

Issue: There is a lot of excitement around the potential of CRISPR genome editing to treat disorders like FA, but the mutations that cause FA also reduce the effectiveness of genome editing.

How will this project address this issue? Researchers will search for potential ways to re-activate genome editing in FA patient cells.

Donor contribution: \$200,000

Person behind the project: Jacob Corn, PhD

Institute: ETH Zurich



Jacob Corn, PhD

ANIMAL MODELS

Project: A Porcine Model for Fanconi Anemia

Issue: A limitation in FA research is the absence of a live model that displays key features of the disease.

How will this project address this issue?

Researchers will create the first large animal model of FA to test potential therapies for people with FA.

Donor contribution: \$50,000

People behind the project: William Fleming, MD, PhD; Markus Grompe, MD

Institute: Oregon Health & Science University



William Fleming, MD, PhD



Markus Grompe, MD

The Fund is committed to supporting research to further our mission of finding new treatments and a cure for Fanconi anemia. Over our 30-year history, we have funded 244 research grants, two clinical trials, and one service grant to 156 investigators worldwide. The total amount of research dollars awarded is nearly \$25 million!

30 YEARS OF FAMILY FUNDRAISING

SINCE FARF WAS FOUNDED IN 1989

624

FAMILIES

have raised funds
for FA research and
family support programs

338 of those families have raised
more than **\$1,000**

206

of those 338 have
raised more than
\$5,000



50K

DONORS

have given to our
cause in honor of
FA families



131K

GIFTS

have been
made to FARF



\$50

MEDIAN

is the median
size gift made

244 **RESEARCH PROJECTS**

2 **CLINICAL TRIALS**

1 **SERVICE GRANT**

have been awarded thanks to your fundraising efforts



1,428

FA FAMILIES

from **59** **COUNTRIES**

have received family
support services



5 **MOST POPULAR FUNDRAISERS**

Holiday appeal letter
Adventure or athletic feat
5K run or walk
Golf tournament
Banquet/party



Cow plop contest
Head shaving & body waxing
All-night video game tournament
Pushing a semi-truck around a track
Company executives in the dunk tank



5 **MOST CREATIVE FUNDRAISERS**

THANK YOU FOR MAKING A DIFFERENCE!



Update from FARF Headquarters

By Mark Quinlan

One of the key operating principles at FARF is fostering collaboration between our various stakeholders, including researchers, clinicians, individuals with FA and families, donors, and staff. This past summer, we worked on several projects that reflect this intentional collaboration. One is the new FARF clinical registry for individuals with Fanconi anemia (see page 8), launched in partnership with the National Organization of Rare Diseases (NORD). After two years in the making, the registry went live this September. This registry will provide researchers and clinicians access to patient-driven clinical data that will aid in our understanding of the medical complications and especially the cancers that affect FA individuals. The registry will provide a pool of patients for upcoming clinical trials, and deepen our understanding of the effectiveness of various therapies. We are now working with adults with FA to enter their data into the registry.

Another initiative is the development of a virtual tumor board (VTB), a group of medical care specialists who meet through a virtual platform to discuss individual cancer cases and determine the best possible treatment plan for the patient. A concern of the FA population is the lack of doctors experienced with adults with FA. The VTB will help address this issue. As is typical with many of our initiatives, this involves coordinating many parties. We are committed to setting up this board and will continue developing the process in the coming months.

We are all aware of the importance of preventing and detecting cancers in people with FA. While the virtual tumor board provides a platform to advise potential treatments, we also need a process to collect tissue samples from individuals

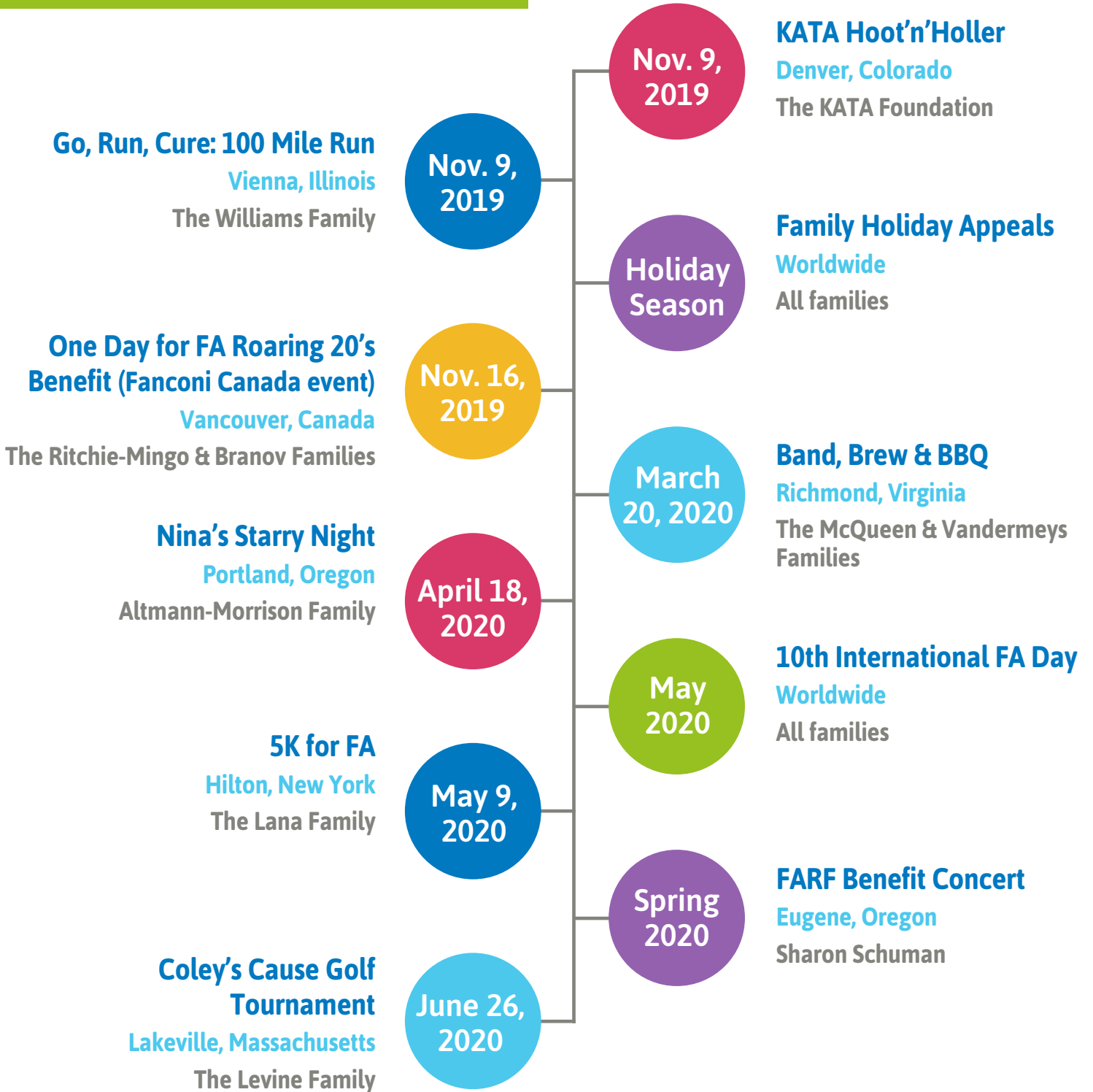
with FA who have surgery. The bottleneck in this process has not been patient willingness or finding researchers and labs to store and analyze the samples. Instead, it has been getting the tissue from the operating room to the lab. FARF has identified a possible solution to this problem. We are working with a nonprofit organization that was created to enable cancer patients to direct their tumor tissue to researchers working on building cancer models. Once the patient has notified them of an upcoming surgery, FARF, along with this organization, will work directly with the hospital to obtain the tissue and transfer it to a lab. We continue to explore what a partnership with this organization will look like and feel optimistic it may provide a solution to this challenge.

Finally, we have improved our process for reviewing and awarding research grants and projects. The change we are implementing involves creating a new Award Review Committee (ARC) made up of researchers, clinicians, and patient/family stakeholders. This group will meet monthly to review proposals and make funding recommendations to the FARF board of directors. As part of the new process, we will end the annual investigator-initiated grant cycle and move to a rolling application process for grant submissions. This new review process will increase efficiency, speed up the award process, maximize staff skill sets, and provide the infrastructure to fund more projects.

Each new initiative we implement benefits the FA community. We would not pursue these efforts if they did not allow us to work more efficiently as an organization, strengthen our partnerships with researchers and clinicians, and ultimately improve the services we provide to individuals with FA and their families. ■

The Fanconi anemia community spans the entire globe, with events in several different locations. The Fund encourages everyone to participate in FA fundraisers. Check this list to see upcoming fundraisers near you! Visit FARF's website to see more events and follow links to find out more information. Do you know of an upcoming fundraiser? Contact us as 541.687.4658 or info@fanconi.org.

UPCOMING FUNDRAISERS



Why Research Matters, continued from page 13

albeit expected descent. We voted for hope.

Now, as with everything else, time will tell. All we can do is be honest with our feelings, empowered in our actions, give Norah every tool we can think of, and take one step at a time.

We are so grateful for FARF, the FA families, and donors for all of the incredible work that's happened over the last 30 years, giving us reasons to hope, ways to connect, and knowledge to share. Thank you. ■



Use of Logo

A reminder to our families with FA: Please use our logo or letterhead only after you have consulted staff at the Fanconi Anemia Research Fund and received approval. This step is necessary to be sure our messages are accurate and consistent, and it helps avoid legal complications. We are happy to collaborate on fundraisers and mailings.

Editors' Note and Disclaimer

Statements and opinions expressed in this newsletter are those of the authors and not necessarily those of the editors or the Fanconi Anemia Research Fund. Information provided in this newsletter about medications, treatments or products should not be construed as medical instruction or scientific endorsement. *Always consult your physician before taking any action based on this information.*



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RETURN SERVICE REQUESTED

Our mission is to find effective treatments and a cure for Fanconi anemia and to provide education and support services to affected families worldwide.

HOW YOU CAN HELP

Donations Online:

Donate via the Fund's website (www.fanconi.org) or through www.networkforgood.org or www.paypal.com

Donations by Phone:

Call us at 541-687-4658 or toll free at 888-FANCONI (888-326-2664) (USA only)

Donations by Mail:

1801 Willamette St., Suite 200, Eugene, OR 97401

Donations of appreciated stock:

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