FAmily Newsletter

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issue 70

In this issue

Robin Lewis, recipient of the 2021 Amy Winn and Christopher T. Byrd Award for Adults with Fanconi Anemia

Update from HQ

Right now, every organization in the world is facing the same question: what will happen when the pandemic is over? We embrace this question with confidence and optimism at the Fanconi Anemia Research Fund (FARF). We know there is light at the end of this tunnel and that FARF will emerge strong. Our commitment to provide a high level of service and invest in the research necessary to improve the lives of those with Fanconi anemia (FA) is unwavering. Throughout the crisis, agility and resiliency have been paramount in continuing to advance research and deliver services. Over the past several months, our efforts and accomplishments ensure continued success into next year.

The past year gave us time to focus intentionally and creatively on organizational development. Since our last newsletter, we have added some new team members. Andrea Ronan, Laura Hefner, Rosie Holcomb, and Jess Stafford have filled critical vacancies in our research, family services, and fundraising teams. Each of these team members adds to the effectiveness of our programs. You can read how each of them is advancing the FARF mission on page 28.

Along with new staff, we have added two new board members: Dr. Carmem Bonfim, an FA transplant clinician from Curitiba, Brazil, and Pedro Ravelo, an FA parent from Chicago (read more on page 27). These two bring incredible experience and insight to our leadership team, which is comprised of FA parents, clinicians, and individuals with experience in business, law, accounting, and fundraising.

In May, Charity Navigator, one of the premier evaluators of nonprofit organizations, notified us that FARF received a four-star rating for the fourth consecutive year. Attaining a four-star rating verifies that FARF exceeds industry standards and outperforms most nonprofits. This exceptional designation from Charity Navigator sets us apart from our peers. It also demonstrates to our stakeholders our diligence and stewardship of the donations we receive. This distinction means that when you ask your communities to support FARF, you can do so with great confidence that their donations are responsibly and effectively used for our mission.

That good stewardship led recently to one of our most exciting research projects. Our collaboration with Stand Up to Cancer, the American Head and Neck Society, Head and Neck Cancer Association, and the Farrah Fawcett Foundation has reached a significant milestone. This group awarded $3,250,000 to a team to study FA and HPV-related head and neck squamous cell carcinoma (HNSCC). We are thrilled with this development and believe the caliber and collaboration of the research team formed gives this project the potential to be game-changing for those impacted by HNSCC.

In addition to research, we also saw growth of international programs this year. We recently awarded a $10,000 grant to The Nate Foundation, a parent-led group that provides education to the medical community and services for FA families in Zimbabwe. They will use the grant to develop educational materials and educate doctors in their country, a significant first step to achieve their dream of one day building an FA clinic to serve those in this region of Africa. More news on international support grants can be found on page 20.

Finally, the highlight of my summer was when I joined FA parent and member of our board of directors, Orion Marx, on his latest Team Bravery adventure. Orion and his crew of friends and family spent five days biking the backroads and trails of Northern Arizona and Southern Utah, visiting six national parks along the way. Orion started doing these athletic challenges many years ago when his daughter Avery was diagnosed with FA. He and the Team Bravery crew have raised over $500,000 through these efforts. Orion knows there are easier ways to raise money; still, he embarks on these challenges because he feels the physical strain they experience during these events is small compared to what individuals with FA experience throughout their lives.

Even as the pandemic has challenged us all, our optimism and commitment are unwavering as we build a better future for individuals with FA. As usual, I am humbled by all who are working, volunteering, and supporting our efforts. You are what makes the FA community great.

Mark Quinlan
Executive Director

SEASON OF *Promise*

How you can help the FA community 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 asking your community for this kind of support can be a challenge. We also know it's so, so important to keep asking. This time of year especially, people look for ways to help, and we've seen the incredible power of communities rallying around our FA family. Here's your end of the year fundraising check-list, an email template, and a sample letter you could send.

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 **https://fundraise.fanconi.org/holidays** 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 2022 with a fundraising report so you know the impact you and your community made.

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

Full letter template on the last page of this newsletter

Dear _____,

How has another year already come and gone? In the blink of an eye, the holiday season is upon us again. This year, (insert short personal update about what your family has been up to in the past year).

As you know, another part of our story is that _____ has Fanconi anemia. Each year at this time, we write to our friends and family to fundraise for the Fanconi Anemia Research Fund (FARF). We do it because FARF feels like an extension of our family. Because they fund research for better treatments and a cure for Fanconi anemia. Because they provide support services to families like ours around the world. Will you help us reach our goal of raising $____ this year?

All gifts, of any size, mean so much to us. If you're able, please check out my fundraising page by clicking this link: _____.

Thanks for reading and helping us raise money for this important cause!

Happy Holidays,

The _____s

TAKEAWAYS FROM THE 2021 Fanconi Anemia Scientific Symposium

The FARF Symposium was held in a virtual four-part series format during the month of July 2021. Fanconi anemia (FA) researchers and clinicians met virtually to share updates on specific topics including gene therapy, bone marrow failure, DNA repair, and cancer. The chairs for each session provided an overview of the research presented and insight on the future of the field.

Gene Therapy & Gene Editing

Co-chairs: Paula Río and Juan Bueren (both at CIEMAT/CIBERER/ Fundación Jiménez Díaz, Spain)

The first day of the Symposium reviewed progress in the FA gene therapy and gene editing fields, with a particular focus on the application of these innovative therapies for the correction of bone marrow failure in FA patients.

Dr. Juan Bueren, a principal investigator of the FANCOLEN I and FANCOLEN II gene therapy trials, shared results from eight evaluable patients that have been treated in the FANCOLEN I trial in Spain. In this trial, six patients have shown progressive engraftment of corrected cells in the bone marrow and peripheral blood, and in two cases, stabilization or even increased peripheral blood cells have been observed at up to five years post treatment with the corrected cells.

Dr. Julian Sevilla (Hospital Niño Jésus) showed the impact of the drug eltrombopag in two patients treated with gene therapy in the FANCOLEN I trial. His data demonstrated that eltrombopag increases the potential for cells to grow in the bone marrow and peripheral blood, suggesting the potential relevance of this drug for improving gene therapy in FA patients.

Dr. Agneiszka Czechowicz (Stanford) and Dr. Jonathan Schwartz (Rocket Pharma) reviewed the progress of the global FANCOLEN II trial. In this trial, FA patients were treated with stem cells corrected by gene therapy in earlier stages of bone marrow failure, as compared to patients in the FANCOLEN I trial. Performing gene therapy at an earlier stage of bone marrow failure enables better collection of stem cells from the patients, which results in higher numbers of infused corrected stem cells. In this trial, six of the seven treated patients who had more than six months of follow up are showing evidence of engraftment. Dr. Schwartz also indicated that five additional patients will be enrolled in the coming months. Importantly, severe side effects have *not* been observed in any patients enrolled in the FANCOLEN I or FANCOLEN II trials.

The second session was dedicated to recent advances in the field of gene editing for FA. This new approach is a recent alternative to lentiviral-mediated gene therapy that allows the targeted correction of disease-associated mutations. As an example of the clinical application of gene editing, Dr. Sandeep Soni (UCSF) presented the results of the first gene editing clinical trial in patients with beta-thalasemia and sickle cell anemia. Because of this genetic manipulation in the hematopoietic stem cells of the patients, most of the treated patients are no longer dependent on transfusions. Although not yet tested in the clinic, new gene editing technologies were reported to efficiently correct specific FA gene mutations in human hematopoietic stem cells in talks presented by Drs. Jacob Corn (ETH Zurich), Branden Moriarty (University of Minnesota), and Paula Rio (CIEMAT Madrid).

To conclude the gene editing session, Dr. David Liu (Harvard) presented impressive experimental studies showing the efficacy

of *in vivo* Base Editing (BE) in a mouse model of progeria (a rare genetic disease that causes children to age rapidly). Base editing is a CRISPR-Cas9-based genome editing technology. In this model, mice treated with the BE technology showed a correction of the clinical hallmarks of the aging disease. Dr. Liu also showed the efficacy of BE for the *ex vivo* correction of the beta-globin (protein in red blood cells) in human hematopoietic stem cells from animal models and patients with inherited blood disorders. The studies presented by Dr. Liu demonstrate the potential of gene editing in correcting anemia and clinical manifestations of other diseases, which are important advancements that may be applicable to FA.

Overall, the session on gene therapy revealed unprecedented results in gene therapy in different diseases, including FA. So far, most of the FA patients treated with gene therapy have shown progressive engraftment of corrected stem cells, with an absence of any conditioning regimen or severe side effects. The long-term follow-up of these patients will confirm the use of gene therapy to prevent, stabilize, or reverse bone marrow failure, and perhaps minimize the risk of hematologic cancers. Additionally, new experimental and clinical data show that gene editing may be a potential therapeutic strategy that can be used in the future to correct specific mutations in FA patients.

Bone Marrow Failure

Co-chairs: Sharon Cantor (UMass Medical School) and Peter Kurre (Children's Hospital of Philadelphia Research Institute)

Speakers and participants from Europe, the United States and Asia shared research outcomes on biology, model systems, and therapy for FA-associated bone marrow failure (BMF) in three separate sessions.

The opening session provided strong evidence that the mechanisms underlying bone marrow failure (BMF) in FA are unlikely to result from loss of DNA repair alone. The first of two keynote speakers, Dr. Filippo Rosselli (Gustave Roussy Institute), discussed evidence that selective loss of FA proteins generates defects in ribosomes (which make proteins necessary for cell growth), already known to cause BMF in related disorders. Work by Dr. Lei Li (Zhejiang University), the second keynote speaker, and Dr. Grant Rowe (Boston Children's Hospital), indicated that the process by which stem cells generate mature cell populations (differentiation) causes cellular stress and loss of stem cells in part by generating aldehyde metabolites.

The talks from this first session suggest that other core cellular functions beyond DNA repair are impacted by loss of FA protein function, and more insight can be expected from the analysis of FA protein function.

Dr. Andre Larochelle, a featured keynote speaker from the National Institutes of Health, discussed early results from an ongoing clinical trial of the drug eltrombopag in FA patients. Previous non-FA studies had suggested a role for the drug in hematopoietic stem cell self-renewal, which was an important finding that helped establish the rationale to develop this trial specifically for FA patients. Exciting early results from the FA trial suggest potential benefit in halting the progression of FA and even restoration of stem cells.

Dr. Larochelle was followed by a series of talks selected from abstracts with more clinical perspectives. Dr. Jean Soulier (Saint-Louis Hospital) showed that certain abnormalities common in FA (gain in chromosome 1q) indirectly reduce the DNA damage response that is considered the critical barrier for preventing clonal evolution to myelodysplastic syndrome (MDS) and acute myeloid leukemia (AML). The results from his laboratory group suggest that gains in chromosome 1q in FA patients may be an important clinical biomarker for progression to MDS.

Dr. Yvonne Behrens (Hannover Medical School) also discussed results on MDS risk-based grouping of FA patients. Her team developed a new system to classify the risk of progression in FA patients based on hematologic, bone marrow, and cytogenetic status. This step wise progression model will hopefully be useful in distinguishing abnormalities that require urgent action from those that can be observed.

A theme throughout the day was that the core problems in FA that lead to BMF remain to be discovered, but ongoing studies continue to expand the potential targets to counterbalance FA deficiency and mitigate BMF.

DNA Repair

Chair: Agata Smogorzewska (The Rockefeller University)

Failure to properly repair DNA strands that become linked by strong chemical bonds (interstrand crosslinks) is a hallmark of Fanconi anemia. Therefore, it is fitting that an entire day of the 2021 Symposium was dedicated to learning more about how FA proteins repair DNA in a meeting titled "When Watson and Crick get linked: origins and repair of DNA interstrand crosslinks." To understand FA as a disease, it is essential to know how these DNA linkages form and how they are repaired. In this year's Symposium, we heard from numerous speakers about discoveries in the FA DNA repair field that have been made in their laboratories over the past year.

What are the sources that cause DNA interstrand crosslinks? We have known for decades that chemotherapeutics cause DNA interstrand crosslinks, but recent work from Dr. KJ Patel's laboratory (University of Oxford) has shown that our cells produce aldehydes, chemicals that react with DNA to form interstrand crosslinks. Dr. Patel showed evidence that formaldehyde produced by bone marrow stem cells can overwhelm the cells and cause DNA damage even with a fully functional FA pathway. Working together with Dr. Minoru Takata at the Kyoto University in Japan, they described how individuals who could not detoxify formaldehyde developed disease very similar to FA despite intact FA proteins. This work is transformative and shows a new paradigm that explains the mechanism of disease in FA patients, and highlights a potential new avenue for therapeutic or prevention strategies for bone marrow failure in FA patients.

Bacteria that colonize our bodies, commonly referred to as the microbiome, are also known to produce chemicals that could cause DNA interstrand crosslinks. Dr. Emily Balskus (Harvard University) and Dr. Silvia Balbo (University of Minnesota) showed that a chemical called colibactin made by certain *E. coli* strains can create DNA interstrand crosslinks. Colibactin has been linked to colorectal cancer, which raises the question of whether this or other bacterial toxins could play a role in head and neck cancer in FA patients. Our understanding of the microbiome in FA patients is still in its infancy, but it is an important area to develop as we work to understand sources of DNA damage in their cells.

How are DNA interstrand crosslinks repaired by the FA pathway? In 2022, we will mark the 30th anniversary of the discovery of the first FA gene, *FANCC*, by Dr. Manuel Buchwald and colleagues. In the intervening period, a collaboration between FA families and scientists resulted in an almost complete list of genes which, when mutated, lead to FA. We have also learned that the proteins coded by these genes work together to repair DNA interstrand crosslinks and studies from many laboratories elucidated the order of events during the repair. The key step during this process is to place a small protein, called ubiquitin, on FANCI and FANCD2 proteins (the ID complex). This ubiquitination reaction is performed by a set of proteins called the FA core complex. Without the ubiquitination of the ID complex, the DNA repair cannot progress properly, causing disease.

In the last two years, the field took a leap in understanding how ubiquitination regulates the function of the ID complex. This was achieved due to a revolution in cryo-electron microscopy (cryo-EM), which allows for taking snapshots of large protein complexes while they are performing their cellular functions. Dr. Lori Passmore (MRC Laboratory of Molecular Biology) described the overall structure of the core complex at last year's Symposium. This year, Dr. Nikola Pavletich, (Memorial Sloan Kettering Center) showed the detailed view of the FA core complex in the midst of ubiquitinating the ID complex. He described how the FA proteins in the core complex are necessary for controlling the ubiquitination reaction that takes place on DNA and results in the ID complex encircling the DNA. The structures he presented are extremely informative and will stimulate more research into the basic mechanism of DNA interstrand crosslink repair.

Cancer

Co-chairs: Susanne Wells and Parinda Mehta (University of Cincinnati)

The final day of the Symposium was moderated by Drs. Parinda Mehta and Suzanne Wells and included three sessions focused on FA solid tumors. One of the biggest challenges in the field of FA is our gap in knowledge on the natural history and biology of FA squamous cell carcinomas (SCC). Understanding the fundamental biological mechanisms of FA SCC is necessary to develop approaches for the prevention and treatment of these tumors. It is more critical than ever that we expand this knowledge and focus on the rapid development of nontoxic methods to prevent and treat FA solid tumors.

Research presented on FA solid tumors focused on three broad topics:

1) surveillance and screening for premalignancies and surgical approaches;
2) new FA SCC mouse models, cancer genomics, and FA protein regulation; and
3) novel immune-based and non-cytotoxic therapies.

The first session of the day featured an overview by Dr. Kutler (Weill Cornell Medical Center) on the clinical challenges in the surgical management of HNSCC in patients with FA. Patients with FA often present at a late stage of disease that requires complex surgeries and have a high risk of recurrence. The two talks that followed Dr. Kutler's were focused on using oral brush biopsy screening as an important way to detect oral cancer early in patients with FA, which would reduce the need for extensive surgery since cancer would be diagnosed at an earlier stage. Madhurima Datta (BC Cancer and University of British Columbia) reported results on a brush biopsy method that focuses on chromosome instability that could be useful for detecting cancer

in high-risk patients, such as those with FA. In past years at the FARF Symposium, Dr. Eunike Velleuer (Heinrich Heine University) has presented her work on a 14-year study that demonstrated the high efficacy and specificity of a brush biopsy procedure followed by conventional cytology and/or DNA cytometry to test visible lesions in patients with FA. At this year's Symposium, she presented work on developing self-examination and education materials, including an interactive mobile app that will empower FA patients and their physicians to engage in early screenings.

In the second session, Dr. Ophir Klein (UCSF) presented a review of the normal oral epithelial tissue wherein FA cancers arise and Drs. Markus Grompe (OHSU) and Ramon Garcia-Escudero (CIEMAT) reported on mouse models of FA SCC. An important new finding by Dr. Garcia-Escudero was the observation that mice that don't express the FANCA gene are prone to pre-malignant and malignant oral lesions. Dr. Agata Smogorzewska (The Rockefeller University) presented genome sequencing data from human FA tumors which indicated a high number of structural variants.

Finally, the third session of the day featured Drs. Robert Ferris (University of Pennsylvania Medical Center) and Stephen Gottschalk (St. Jude's) who reviewed immune and cellular therapies by checkpoint inhibition and CAR-T cells, respectively. Anecdotal reports of checkpoint inhibitor use in patients with FA with SCC are available, but more objective data from well-thought-out prospective clinical trials will aid in clearly defining their role. Such therapies provide hope and may turn out to be effective cancer treatments in FA.

Dr. Dipak Panigraphy (Harvard) reported on studies focused on the role of inflammation in FA cancer as a stimulator of immunotherapy. Drs. Gary Kupfer (Georgetown), Khashayar Roohollahi (Amsterdam UMC), Muhammad Rahman (Barts) and Jordi Surrallés (St. Pau Hospital) presented data supporting potential cancer therapies via inhibitors of mitotic regulators, immune response pathways, and epidermal growth factor receptor (EGFR) signaling. Dr. Jordi Surrallés is currently planning to develop a clinical trial to test the EGFR inhibitor, afatinib, as a treatment for FA SCC.

Tremendous progress has been made regarding early diagnosis, development of novel mouse models, and identification of molecular targets and therapeutic approaches for FA SCC. A common theme that emerged from the research presented in this session and in panel discussions centered around the critical need to identify new targeted strategies that are biology driven and can be safely used for prevention and/or treatment of these cancers in individuals with FA.

In Loving Memory

Wanda Doms
7.7.1978 – 6.2.2021

Viyan Senthilkumar
5.4.2017 – 4.21.2021

Dawn Sanguedolce
11.22.1967 – 7.11.2021

Samuel Boudreau
10.28.1996 – 7.12.2021

Zachary (Zach) Brinkmann
6.15.2006 – 8.13.2021

Christian Collins
8.25.1993 – 8.27.2021

Emiliano Andrade
11.22.1994 – 9.12.2021

Scientist Spotlight

Name: **Lindsey Romick, PhD**

Institution: **Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio**

Area of expertise: **Metabolism in Human Disease and Cancers**

My work:

My research focuses on understanding the underlying metabolic dysfunctions in persons with Fanconi anemia that lead to stunted growth, an inability to gain weight, and/or abnormal subcutaneous fat storage, and increased risk of both onset and progression of cancers. The goal of this work is to pinpoint what metabolic abnormality is leading to the above conditions and implement interventions, such as customized nutritional plans and/or supplementation with needed nutrients and enzymes that may be absent or poorly used in persons with FA.

What motivates me to work on FA:

The FA community motivates me every single day. I was lucky enough to be invited to Camp Sunshine in June 2012 as a brand new postdoc in Dr. Susa Wells' lab. I had only heard of Fanconi anemia for the first time a few months earlier. When I left camp that year, I knew that I would dedicate my career to understanding this disease. I fell in love with the individuals with FA, with the families, with the community and almost 10 years later that passion to help this population has only grown stronger.

When I'm not in the lab, you could find me:

I spend nearly all of my free time with my daughters, Lily (10) and Annie (6). We love being outdoors and exploring nearby nature centers and parks and finding nearby lakes and rivers to paddle our kayaks and standup paddleboards on.

Anything else you want FA families to know?

Thank you for your unwavering support of FA researchers like myself. Thank you for your endless strength and for always being willing to ask us the tough questions. It has made me a better scientist and a better human being.

Facing a cancer diagnosis?

The FARF Virtual Tumor Board is here to help

What is the FARF Virtual Tumor Board?

The Fanconi Anemia Research Fund (FARF) Virtual Tumor Board (VTB) is a panel of physicians experienced with treating patients with Fanconi anemia (FA) who volunteer their time to discuss difficult FA solid tumor cases and offer treatment guidance. The specialists on the panel have expertise in otolaryngology, hematology oncology, radiation oncology, and medical oncology.

The VTB was developed to provide support to individuals with FA and their treating physicians, who may have less experience with treating cancer in individuals with FA. Members of the FARF VTB meet virtually with patients' treating physician(s) to review cases and provide input for treatment, from an FA-centric viewpoint. This process allows continued learning on how to best treat cancers associated with FA, which in turn may help others with similar diagnoses.

How do I know if this service is for me?

Early intervention is essential when treating solid tumors in individuals with FA. If you are undergoing surveillance for a potential cancer, but have not yet been diagnosed, please contact FARF so that we can be poised to expedite the services of the VTB in the event of a diagnosis.

You can only have your case reviewed by the VTB if you have been diagnosed with cancer. The VTB does not convene for cases in which individuals are undergoing surveillance or testing for solid tumors, but have not yet been diagnosed.

When should I request VTB services?

The FARF VTB program is most useful if you discuss the program with your treating physician immediately following a solid tumor diagnosis. If you and your treating physician agree that soliciting advice from the VTB would be advantageous for developing a treatment plan, either you or your treating physician should contact FARF directly. We will then coordinate with your physician to set up a VTB meeting to review your case.

Your FARF contact is Andrea Ronan: andrea@fanconi.org or 541-687-4658 ext 402.

Do I participate in the VTB meeting?

The VTB meeting is a physician-to-physician discussion; patients do not participate. FARF will not provide a formal report on the meeting outcomes to you. Any treatment guidance provided by the VTB will be relayed to you by your treating physician.

Do I have to pay for this service?

The physicians on the FARF VTB generously volunteer their time for this service, which FARF offers at no cost to you.

FANCONI ANEMIA RESEARCH FUND

FANCONI ANEMIA VIRTUAL TUMOR BOARD
FANCONI ANEMIA RESEARCH FUND

Contact FARF Clinical Science Program Manager Andrea Ronan | andrea@fanconi.org | 541-687-4658 ext 402

Current CLINICAL TRIALS and RESEARCH OPPORTUNITIES

New treatments and therapies for people with Fanconi anemia are not possible without research. Listed below are current clinical trials and research opportunities available. 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. Please contact us at info@fanconi.org or 541-687-4658.

Clinical Trials for Hematologic Issues

Antibody-Based Conditioning with TCRab T-cell/CD19 B-cell Depleted Allogeneic Transplantation for Fanconi Anemia Patients with Cytopenias

Stanford University, Stanford, CA | Active, currently recruiting participants

Open to Fanconi anemia patients of all subtypes, ages 2+. **Eligibility includes having developed cytopenias (reduced blood cell counts) and not having an HLA-identical matched sibling donor for bone marrow transplant (BMT).** Patient must not be on other experimental therapies at the time and not have active cancers or concerns for high-risk bone marrow disease.

The objective of the study is to prepare the patient's body before a stem cell transplant by using an antibody-drug instead of radiation/chemotherapy to make transplants safer. To prevent rejection of the donor cells, prior to BMT, patients will be treated with standard immune suppression and an antibody-drug, JSP191, in place of genotoxic irradiation or busulfan treatment. Blood stem cells are collected from healthy donors and purified to remove problematic T-cells. These healthy stem cells are then given to the patient by intravenous infusion.

Contact: Bone Marrow Failure Program Team | 650-497-8953 | bmf@stanfordchildrens.org

Stem Cell Transplant From Donors After Alpha Beta Cell Depletion in Children and Young Adults

Stanford University, Stanford, CA | Active, currently recruiting participants

Available to individuals ages one month to 60 years old who are deemed eligible for allogeneic Hematopoietic Stem Cell Transplant (HSCT) per institutional guidelines. See study information for additional inclusion and exclusion criteria.

In this study, the participant will undergo a stem cell transplant using donor cells that have been manipulated through an investigational device (CliniMACS® TCRαβ-Biotin System and CliniMACS® CD19). The purpose of the study is to improve the safety and efficacy of allogeneic HLA-partially matched related or unrelated donor HSCT when no matched donors are available. Participants will be followed for outcomes for two years.

Contact: Alice Bertaina | scgt_clinical_trials_office@lists.stanford.edu

Quercetin in Children with Fanconi Anemia; a Pilot Study

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

Available to Fanconi anemia patients of all ages who are able to take medication by mouth. See study information below for exclusion criteria. **This trial is for individuals who are pre bone marrow 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..

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

Eltrombopag for People with Fanconi Anemia

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

Eligibility includes people with Fanconi anemia, ages 2 years or older, weighing greater than 10kg. **This study is for individuals with reduced blood cell counts defined as clinically-significant cytopenias.** See study information for details regarding cytopenias as well as exclusion criteria.

Based on clinical and pre-clinical studies, the team hypothesizes that Eltrombopag (EPAG) will improve peripheral blood cell counts in patients with FA, thus positively affect morbidity and mortality. Of particular interest for patients with FA is the observation that EPAG also improves the repair of double strand DNA breaks, a mechanism that is impaired in patients with FA. The objective of this study is to determine if EPAG is effective in FA patients and the length of treatment needed to improve blood counts.

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

Long Term Effects On Recipients of Hematopoietic Stem Cell Transplantation

Stanford University, Stanford, CA | Active, currently recruiting participants

Open to individuals who are either scheduled to receive or have completed a Hematopoietic Stem Cell Transplant (HSCT). Those with HSCT completed at another institution other than Lucile Packard Children's Hospital (LPCH) are eligible although follow-up long-term

care must be transferred to LPCH. Participants will be seen thorough the late-effects clinic in the Pediatric HSCT Clinic. Participants who have relapsed from a malignant diagnosis post HSCT and are not being worked-up for a new HSCT are not eligible.

The goal of the study is to establish systematic follow-up care for HSCT recipients by collecting data and tissue samples and creating a comprehensive database to demonstrate survivor's clinical status through their life span.

Contact: Nivedita A Kunte | 650-497-2038 | nkunte@stanford.edu

Cancer trials: chemoprevention

Quercetin Chemoprevention for Squamous Cell Carcinoma in Patients with FA

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

Available to Fanconi anemia patients ages 2 years and older, who are able to take medication by mouth. See study information for exclusion criteria.

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.

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

Registry-based trials

Fanconi Anemia Registry

Fanconi Anemia Research Fund | currently recruiting participants

Open to all individuals with Fanconi Anemia.

The Fanconi Anemia Registry is a participant-driven resource that empowers and unites the FA community through shared knowledge. Registry participants can complete surveys about their own disease experiences. Through the registry we can track cancer cases in individuals with FA as well as treatments. The overarching goal is to assist the FA community with the development of recommendations and standards of care and to be a resource for researchers interested in FA.

Contact: Andrea Ronan | 541-687-4658 | andrea@fanconi.org

Cancer in Inherited Bone Marrow Failure Syndromes

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

Open to all Fanconi anemia patients, their first-degree relatives defined as siblings (half or full), biologic parents, and children, and grandparents.

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.

Contact: Neelam Giri | 240-276-7256 | girin@mail.nih.gov

Natural History of FANCD1/BRCA2

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

This is a subgroup within the National Cancer Institute Cancer in Inherited Bone Marrow Failure Syndromes, listed above. It has been 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. This subgroup was created in order to determine the natural history of patients with FA associated with mutations in FANCD1/BRCA2.

Contact IBMFS Study Team | 1-800-518-8474, or email NCI.IBMFS@westat.com

International Fanconi Anemia Registry (IFAR)

The Rockefeller University, New York, NY | currently recruiting participants

Open to all individuals with Fanconi anemia. Enrollment required for tissue donation through The Rockefeller University.

The purpose of the IFAR is to study the nature, diagnosis, and treatment of individuals with FA. Information collected in this study will help researchers better understand FA and be able to better diagnose and treat the condition. We enroll patients at any stage of the disease, but many recent studies are focusing on understanding cancer development in FA patients. Please reach out if you have been diagnosed with cancer or pre-cancer lesions.

Contact: Agata Smogorzewska | 212-327-8612 | fanconiregistry@rockefeller.edu

Other FA Systems

Nutrition & Metabolism in Fanconi Anemia

Children's Hospital Medical Center, Cincinnati, OH

This study is actively recruiting and consenting participants. They must be 12+ years of age and those with diabetes or need for insulin are ineligible for the study. This clinical trial uses tracing experiments to show how efficiently and effectively individuals with FA can utilize glucose versus fat and protein for energy. Studies have shown that individuals with FA break down fat and muscle at a quick rate, making it difficult for them to maintain weight or build muscle. Studies have also shown that individuals have low levels of Carnitine, known to combat DNA damage. Poorly functioning metabolic systems influence physical appearance, immune function, host defense, and brain energy. Also to be considered are the equally significant impacts on the psychological, social, and emotional wellbeing of individuals with these metabolic challenges. The results obtained from this trial could lead to treatment options that combat body mass index issues, including those pertaining to muscle mass, and could positively impact the general psychological and emotional resilience and wellbeing of the FA population.

Contact: Lindsey Romick-Rosendale | 513-517-0256 | Lindsey.Romick-Rosendale@cchmc.org

Turning My Hardships into An Epic Journey

By Thomas Paris

Hello, my name is Thomas Paris. I'm 42 years old and live in Paris, France. I was diagnosed with Fanconi anemia (FA) at age 24 with a FANCA mosaic mutation. Since childhood, I knew I was ill but didn't know how ill until I was diagnosed with FA. I'm proud to say that I am one of the oldest people with FA in my country as well as the tallest man with FA in France, standing at 1.72m (5ft 6in).

I went through a bone marrow transplant (BMT) at 32 and have written two books to share my story. Because I didn't know if I would survive the transplant, my first book really became my baby. The first book is about being diagnosed with bone marrow failure and about my transplant experience. After I finished this book, I shared it with family and friends so they could better understand what I experienced. My second book is a reflection about the graft-versus-host disease I struggled with following my BMT.

Since my transplant, I've had around 30 cancers – many of them on my head - and several surgeries. I've had cancer on my lip which required my stomatologist to construct a new lip. One of my worst cancers was anal cancer. It was painful for me to use the toilet and I ended up needing extensive surgery.

I also had a spot on my brain that was found to be non-cancerous following a biopsy. It did cause inflammation, however, making it impossible for me to walk and I spent three months in the hospital. I developed amnesia and had to write everything down to remember it. I also

Australia 2009

Crete 2010

Iceland 2012

began not to remember people around me. Doctors used plasmapheresis (the removal, treatment, and return of blood plasma from and to the blood circulation) to try and cure me. Slowly, I partially recovered my ability to walk but I still cannot run, and I struggle to stay balanced.

Unfortunately, I couldn't go back to work and my girlfriend at the time broke up with me because of my disability. I know that cancer will most likely return, but I'm keeping a positive attitude as I saw people in much worse situations around me in the hospital. I saw people confined to their beds as quadriplegics and I knew I shouldn't complain about my situation. Every time I have a biopsy, it hurts, but I also remind myself "no problem, I can get through this pain, the future will be better soon, and I will not suffer."

I am passionate about traveling and I hope to be able to travel more in the future. I try to make the best out of hard situations. It is not always easy to do, but I realize that without all these things I've been through, I wouldn't be the good man I am today. I'm more empathetic than before and more sensitive to other people's problems. We all have unique life paths and obstacles to overcome. Sometimes we fall, but we also get up, heal ourselves and continue with life. Carrying FA on your back is a burden that is difficult to live with. FA hurts. It's long and painful, but this pain is what makes us remember the parts of life worth living. The challenges we face make our lives rich and even transform them into an epic journey.

Sicile 2014

"We all have unique life paths and obstacles to overcome. Sometimes we fall, but we also get up, heal ourselves and continue with life."

ROBIN LEWIS
receives the 2021
Amy Winn & Christopher T. Byrd Award for Adults with Fanconi Anemia

Congratulations to Robin Lewis, recipient of the 2021 Amy Winn and Christopher T. Byrd Award for Adults with Fanconi Anemia. Robin was born in Pretoria, South Africa and was diagnosed with Fanconi anemia (FA) at the age of 25. Despite the challenges of FA, Robin believes that FA has given him a new chance at a life filled with a positive outlook on his goals, attitude, perspective, and the planet. "I hope to inspire others to live a life full of love and appreciation."

To enact this mission, Robin and his wife, Jolandie, started Numinous Expeditions in 2017. Numinous Expeditions is a nonprofit that carries out environmental, humanitarian, and animal welfare projects with a focus on ethical solutions and long-term outlooks. Their goal is to visit as many countries on the African continent as possible in their home-built expedition truck "Betsy". In each country, they aim to complete a project catered to the needs of the local community. To date, they've completed nine community projects. Their latest project is called Eco-Flow and aims to educate 1,000 school children in rural South Africa about the benefits of sustainable farming, health, and dietary practices.

"Our focus embodies the three elements that exist on our beautiful planet: humans, animals, and nature. They can't always be in harmony, but it is our pleasure to attempt it. Our projects tend to come our way naturally, and I truly hope we will be able to make a lasting impact on the world around us."

The Winn/Byrd award helps Robin and Jolandie start the next chapter of their journey. Importantly, it's allowed them to add a much-needed housing section to "Betsy". If you would like to learn more about Numinous Expeditions' work or provide support, please visit their website at **www.NuminousExpeditions.co.za**

Robin is dedicating their road trip across the earth to FA families and the FA community. He will help educate others about FA and hopefully motivate others with serious health conditions to take action by sharing his own story. His positive outlook on life and drive to improve the lives of other people is truly inspiring. "I look forward to sharing our venture with the FA community for years to come and wish all of you good health and true happiness."

Robin's Acceptance Speech

I'd like to start off by saying what a great privilege and honor it is to have been selected as the recipient of the 2021 Amy Winn and Chris Byrd Award for Adults with FA.

It is unfortunate that I cannot thank you in person, but hope to express my gratitude from afar.

I've been overwhelmed by the thought of receiving this award in memory of Amy Winn and Christopher Byrd's inspiring lives and outlooks. Their positivity and love for life is what we strive towards, and their stories truly inspire many to reach out and appreciate the gift that each day represents.

Fanconi anemia swept in and rocked our lives to the core. For many of you it has been a source of great heartache and pain, and the suffering you have endured is only testament to your strength. For us, it has also inspired a second chance. FA brought an abrupt and eye-opening experience to our lives. We could not have imagined our journey up to this point. Although it has been very challenging and painful at times, we now see beauty in our everyday lives that I can only describe as an innate gift.

Since my journey started, FA has changed my perspective and saved a lifetime of chasing a false sense of happiness. My mortality has been a motivating factor for inspiring change in how we appreciate all of creation, and what purpose our lives have.

I could not have achieved this highlight of my life without the love and support of my wife, friends, family, and our Creator. I hope to live up to this award, and hope to inspire others to live a life full of love and appreciation.

We have to ask ourselves what meaning our lives have to others, and to ourselves.

Robin, Jolandie, Betsy. The award funds will be used to complete their home on wheels.

Do you chase your dreams? Do you stare at the stars, and smile at the sunsets? To be loved does not compete with being able to love. Having a passion for compassion can sometimes put you in challenging situations, but is almost always worth it. A couple of years ago I made an oath that would shape our lives and decisions. It would ultimately lead to this moment, and is proof that the universe conspires with you when you follow your destiny without hesitation or fear.

I intend to stick to my oath, and I'm in the fortunate position to have a wife who would risk a full life, to make the most of my possibly limited one. Our mission will take us across 57 countries throughout the next 20 years. Our goal is to complete one meaningful project in every country we manage to reach. Our focus embodies the three elements that exist on our beautiful planet. Humans, animals, and nature. They can't always live-in harmony, but it will be our pleasure to attempt it. Our projects tend to come our way naturally, and I truly hope that we will be able to make a lasting impact on the world around us.

We would also like to dedicate our planned road trip across the earth to Fanconi anemia families and spreading FA awareness. Some of the biggest supporters in our venture have been individuals from the FA community. I hope to accomplish what seems to many as impossible, but more importantly, to inspire some to reach for it even though the odds are against them!

This award will make it possible for us to complete most of the work we need to start the next chapter of our journey. It enables us to gain the supplies needed to finish building our expedition truck – Betsy.

To Lynn Frohnmayer, I'd like to thank you for all of the years of exceptional work you have dedicated towards giving hope to others. I commend you as well as all of the other FA parents and families that have endured suffering beyond my comprehension. All of the FARF staff and researchers that have found passion in their work, it gives FA warriors hope that the right people are on the case, and finally to the donors that support the Fanconi Anemia Research Fund, you have chosen to put your trust and funding into a noble cause, and through your contributions have made a true difference in the lives and futures of many.

I look forward to sharing our venture with the FA community for years to come, and wish all of you good health and true happiness. Thank you!

Treatment: 2018 transplant in isolation room.

Robin's goal was to summit Table Mountain in Cape Town one year post transplant, which he managed to do.

Isolation ward mural project: this photo was taken with Lithemba and his mother in 2020 after completing our eye candy project. Lithemba had FA and was the first patient to receive treatment in one of the painted rooms. Unfortunately, he did not survive the transplant.

Journey of a Thousand

By Sylvia Sanyanga

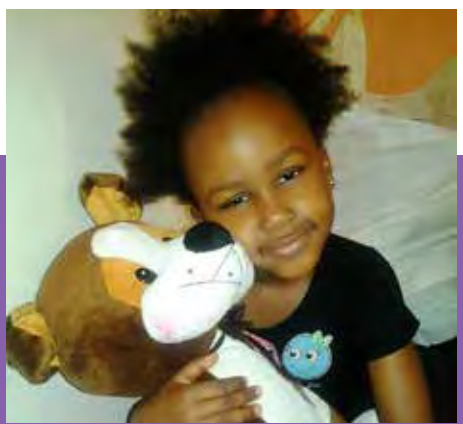
Our family's story

Our journey began in 2013, when our daughter Natasha was diagnosed with Fanconi anemia at the age of five after experiencing bone marrow failure. This was all new to us and very confusing. We did not understand how our daughter who was seemingly healthy had a life-threatening condition. We would later find out as we learned more about the condition that she was born with it. We were advised that she needed a bone marrow transplant. Quite sadly, there was no medical institution that could perform bone marrow transplants in our country and there still is not one. As we conducted further research, we discovered that not only was a bone marrow transplant a life-threatening procedure, but it was also very expensive. My husband, Eddie, and I decided that we were going to do everything within our power to give our daughter a fighting chance.

We began a fundraising campaign to raise $100,000 (USD) which was required for the transplant in India ($45,000 for the transplant, $35,000 for the donor and $20,000 for airfares and living expenses in India for the entire family for six months). With only two days before the scheduled day of departure to India, having raised $23,000, we received incredible news that a donor who wanted to remain anonymous had paid for the transplant in full ($45,000) and was ready to pay the $35,000 if any of us were not a match for Natasha. They also advised us that we could use whatever we had raised for travel and living expenses while in India. What a MIRACLE! Natasha had her life-saving treatment in India with no major complications and her brother, Raymond, who was three years old at the time, was the donor, a perfect 10 out of 10 match.

In 2015, we were blessed with our third child Nathan (meaning God's Gift). Our adorable little boy was born with Fanconi anemia, bilateral club hands, an absent radius, no thumbs, an absent right kidney, and severe hearing loss. When he was only two months old, he had to undergo surgery as he had a right inguinal hernia. At six months he had corrective surgery (centralization of his right hand) and a year later, when he was one and a half years old, he had another one for his left hand. The surgeries Nathan underwent were supposed to cost us a total of about $40,000 and I am happy to share that Nathan benefited from free surgeries performed here in Zimbabwe by a team of plastic surgeons from the University of San Francisco. We will forever remain grateful and indebted to them for selfless acts and amazing work. Nathan has full use of his hands and is thriving, a budding artist with great talent.

In 2018, when Nathan was three and a half years old his bone marrow failed, and he underwent a bone marrow transplant. He endured numerous infections such as a terrible adenovirus (a common virus that causes a range of illness and cold-like



Natasha pre-transplant

Natasha (left) post-recovery

Raymond post harvest

Miles Begins with One Step

symptoms), battled with graft-versus-host disease because his brother Raymond was a haplo-identical match (half match), and Nathan had to be in ICU (where at one point we were told to prepare for the worst). We kept hope and faith alive and are happy to share that this little boy fought with such bravery and resilience. We are happy to share that our children Natasha, Raymond, and Nathan are in good health, thriving, doing well in school and continue to inspire so many people, young and old.

You can learn more and follow Natasha and Nathan's journey on Facebook (**https://www.facebook.com/sylviasanyanga1/**)

Paying it forward

Following our ordeal, Eddie and I agreed that no parent should ever have to go through what we went through, so we founded and registered The Nate Foundation, an organization that supports children with Fanconi anemia and related conditions, their families, and their caregivers. When we faced challenges finding a preschool that would accept our son Nathan, we set up Caterpillar Clubhouse, a preschool that is open to children with special needs and fosters inclusive learning in mainstream school.

We are glad to share that our story has been a source of hope and inspiration to other patients with FA and their families. Through our organization, we have been able to help some children go to India for bone marrow transplants. We also provide psychosocial support and work together with medical personnel in our country to raise awareness on FA in Zimbabwe. We have done this work with minimal donor support as our government and most people in our country have limited financial capacity. However, we are excited that we received our first ever grant of $10,000 from FARF August 2021. This huge show of support will ensure that we continue to raise awareness of FA and provide support to patients, their families, medical personnel, and health facilities.

When we count our blessings, we count our children twice. What an honor it is to have beautiful, brave, and loving children. They have taught us so much, the biggest lesson being that no matter what life throws at you, fight. There is a blessing in the storm. Who knew that our story of pain and struggle would turn into a tale of beauty.

We have been called to serve, and diligently we shall. The greater vision is an Aplastic Anemia Treatment Centre, with a bone marrow unit, a learning center for children with special needs, research facilities and a resource library. Our journey of a thousand miles began with one step. We cannot wait to see how it will unfold for us, Zimbabwe, and Africa at large.

Learn more about us:
The Nate Foundation: **www.facebook.com/TheNateFoundation**
Caterpillar Clubhouse: **www.facebook.com/CaterpillarClubhouseZW**

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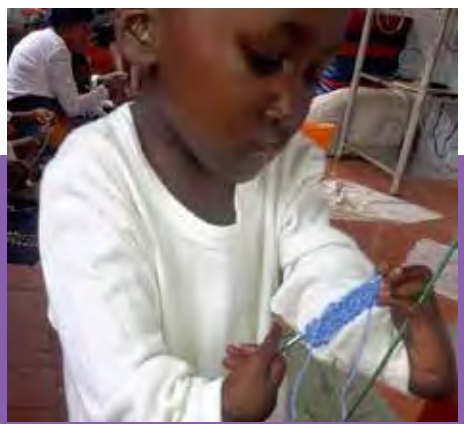
Nate (far right)

Nathan and Eddie



Nate knitting

What is Self-Care, How You're Already Doing It, and How It Can Improve Your Life

This text is from the Taking Charge of Your Survivorship website, a tool developed by the University of Minnesota to support individuals living with chronic illness and their loved ones. Find many more great articles and resources at ***www.takingcharge.csh.umn.edu/survivorship***

> **"Self-care seems like a buzz word that people are getting sick and tired of. In reality, you probably do and value some form of self-care every single day, whether you think of it that way or not."**

When people think of self-care, they can get a little cynical. It gets a reputation for being shallow or something that only the privileged get to do. The reality is, everyone does some basic self-care, or you probably wouldn't be here.

According to the International Self Care Foundation, it is estimated that 70-95% of all illnesses are managed without the intervention of a doctor. That means that self-care is usually the first treatment for everyday health conditions. In the case of living with chronic illness, you are tasked with doing a great deal of self-care just to keep up with your healthcare team's recommendations.

Self-care includes:

Hygiene

- Washing hands
- Regular bathing
- Cleaning your teeth
- Wearing clean clothing

Physical activity

- Dancing
- Climbing stairs
- Walking
- Yardwork
- Yoga

Avoiding risky behaviors

- Smoking
- Tobacco products
- Alcohol abuse
- Illicit drugs
- Not wearing a seatbelt
- Not using a helmet

Sleep

- Getting 6-8 hours a night
- Developing a sleep routine
- Sleep during nighttime
- Get physical activity during the day to sleep better at night

Nutrition

- Eat a variety of foods
- Choose colorful foods
- Mostly fruits and vegetables
- Know the ingredients of the foods you eat
- Eat consistently throughout the day

Seeking care and support

- Scheduling regular medical appointments
- Asking for help
- Talking to a mental health professional
- Accepting help

An important, and often overlooked, aspect of self-care, is practicing the ability to really listen to your body and what it needs at any given time. In other words, making the proper self-care diagnosis. It's so easy to get sidetracked because you get bombarded with social media posts and advertisements about self-care products, plans, or other "must haves." If you're feeling down or simply a little off, it's easy to fall into these traps. Instead, first try to slow down. Find an activity you enjoy and do that to clear your head. Once you get to a place where your mind isn't racing, ask yourself what it is that you need. You can do this in small ways and in big ways.

For example, if you're trying to multitask and hurry to get ten things done around the house, and you find yourself very irritable, it's worth taking a step back and assessing the situation.

Take a moment to ask yourself

- Is there excess background noise that's making you irritable?
- An abandoned TV blaring in the next room that could be turned off?
- Are you hungry? Thirsty? Tired?
- Are you running late? Or forgetting something?

Once you've identified the top 1-3 things that are contributing to your irritability, you can make some simple adjustments to your situation and reset.

A bigger picture example is taking time to reflect and plan, or take control of your time and energy. For this, you can try daily journaling. You don't need to do a lot of writing, just make bulleted lists of the things that drain you and the things that fill you up. Simply taking the time to reflect helps, but writing it down helps you identify patterns over time.

People really envision things like spa days, salads, and workouts when they think of self-care. But important mental and emotional health strategies are rarely ever mentioned. Setting boundaries is an important example. Setting boundaries with your time, energy, and emotional expenditures is often the most impactful self-care step you can take. It isn't easy at first, especially because setting boundaries often involves other people, but it gets easier over time. Remember, you don't have to explain yourself and you definitely don't have to apologize.

Resilience

Resilience is your ability to bounce back after something tough happens. Building resilience is one of the main motivators for practicing self-care. Every act of self-care you do is like making a deposit into your resilience savings account. You can't self-care your way out of a crisis, but if you've spent years investing in your resilience savings account, you will be better prepared to handle any crisis life throws at you, and you will recover more quickly.

Instead of falling into the "all or nothing" mindset, try to praise yourself for every act of self-care you do. Instead of beating yourself up because you didn't work out, you might think, "my body must have really needed that extra hour of sleep this morning." And then strategize how you might fit that workout in later.

Applying it to your life

Self-care doesn't have to be a monumental event scheduled into your day. Instead, it could look like: "I have 25 minutes until my next meeting. What can I do for 20 minutes? Walk, stretch, pet the dog, eat? What would energize my body and my mind?"

Self-care looks different for everyone. It is impacted by things like your baseline physical and mental health, your responsibilities both inside and outside the home, your social determinants of health, and by the national and global crises such as the pandemic and racial injustice. The most impactful forms of self-care don't cost anything. Slow down, if even for just one moment, tune in, reflect, listen, act.

Impact Around the World

United Kingdom and The Netherlands (2019)

This project aims to unite the existing FA support groups across the 23 member states of the European Union into an umbrella group that increases collaboration between countries and partnerships with EU healthcare networks and pharmaceutical companies. FA Europe connects patients, clinicians, and scientists, and acts as a facilitator for fundraising, sharing best practices and collaborating on research efforts.

Mexico (2020)

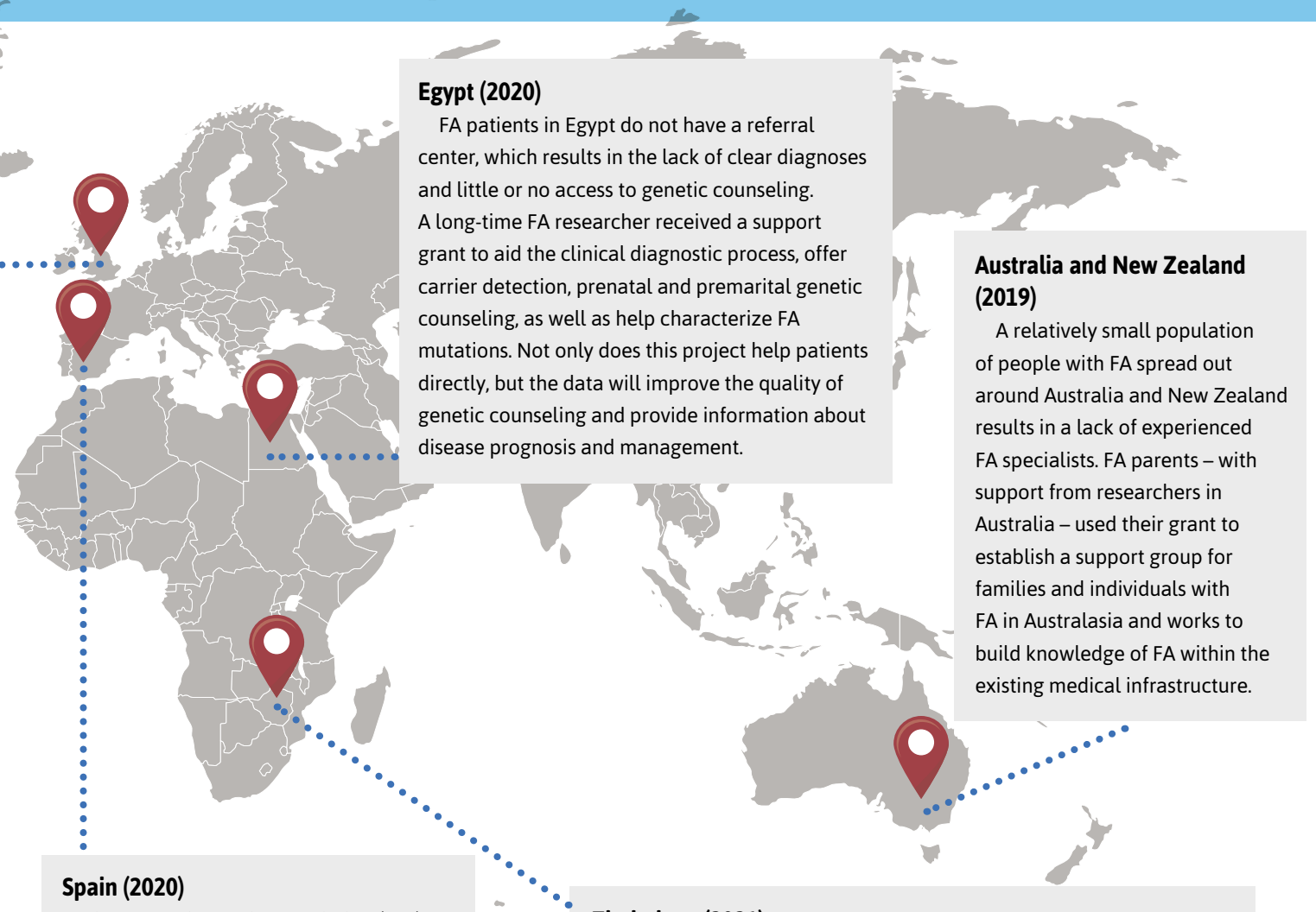
The opportunity for patients and families to meet in person is the first step to building a vibrant and organized FA community in Mexico. The aim of this project is to hold the very first meeting for FA patients in Mexico. Educational sessions tailored to target patients with FA and their families will be held to provide reliable and up-to-date FA information and research participation opportunities. The meeting will take place once it is safe for participants to gather.

Dominican Republic (2019)

Un Corazon por Fanconi, a foundation started by an adult with FA, Ana Tabar, received a grant to develop a diagnostic protocol for FA patients in the Dominican Republic (DR), provide support services for FA families, and build organizational capacity. With help from experienced FA researchers in Mexico, the team in the DR is working to form a network of doctors who can learn about the diagnosis and management of FA.

While 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 in 2018. Each year, FARF awards up to five $10,000 grants for one-year projects that address the needs of the global FA community. These needs include access to medicine, doctors, and facilities; organizational and infrastructure needs and access to family support services.

So far, seven international support grants have been awarded and programs are underway in nine countries as a result.



Egypt (2020)

FA patients in Egypt do not have a referral center, which results in the lack of clear diagnoses and little or no access to genetic counseling. A long-time FA researcher received a support grant to aid the clinical diagnostic process, offer carrier detection, prenatal and premarital genetic counseling, as well as help characterize FA mutations. Not only does this project help patients directly, but the data will improve the quality of genetic counseling and provide information about disease prognosis and management.

Australia and New Zealand (2019)

A relatively small population of people with FA spread out around Australia and New Zealand results in a lack of experienced FA specialists. FA parents – with support from researchers in Australia – used their grant to establish a support group for families and individuals with FA in Australasia and works to build knowledge of FA within the existing medical infrastructure.

Spain (2020)

The Fanconi Anemia Foundation (FAF) received a grant to support their efforts to increase fundraising and mobilize affected families as fundraisers. This grant enabled FAF to partner with a digital marketing company that developed a fundraising and communications strategy and plan. FAF continues to build their supporter base and increase their income to support research and FA families in Spain.

Zimbabwe (2021)

The Nate Foundation was founded by parents of two kids with FA in Zimbabwe. They recently received an FA support grant to address the lack of information about FA in their country and improve access to support and information for patients. First, they will create a support group for individuals with FA and their families. Next, they will prepare educational information and give seminars to medical institutions. Finally, they will partner with healthcare practitioners and local government to host an educational campaign to spread awareness about FA among the public and within the medical community.

FAMILY FUNDRAISING SHOUTOUTS

Turning sandcastles into sand dollars

The most unique fundraiser of the year award goes to the Hawkshaw Family from Australia! Amelia, who lives with FA, her husband, Zach, and her family built a giant sandcastle to celebrate FA month. The more money they raised, the higher and more complex the sandcastle became. By the end of their campaign, the Hawkshaws built a sandcastle over 6.5 feet tall, complete with a staircase and a bridge. Not only did they set a record for the tallest sandcastle in family history, but they raised over $2,500! Thank you, Hawkshaw family, for fundraising in such a creative way!

Callie jumps in to fundraising

After receiving her FA diagnosis last year, 22-year-old Callie Toal quickly found the FA community. When asked what motivated her to raise funds, Callie answered that she was happy to "support research and others like her, while also educating others unaware of this disorder." She jumped right in to start fundraising during FA month and raised nearly $10,000! Callie, thank you for bravely sharing your story and supporting FA research!

Spreading awareness through gaming

FAdult Council member Daniel Kold hosted a 24-hour live videogame stream to raise funds and awareness for FA. While Daniel played, 450 viewers learned about his experience with FA and the support services that FARF provides. In addition, he raised over $300 for FA research and support services. Thank you, Daniel, for showing us how a hobby can become a fundraiser.

Campout for a cause

This summer, 16-year-old Ryan Healey camped out to raise funds for FA research in memory of his baby sister Elizabeth, and in honor of his 12-year-old brother Aidan. He collected donations from friends and family, including his grandfather, a big supporter of the campout who passed away earlier this year. Ryan raised $3,000 for research and services. Thank you for standing up for a cause you care about, Ryan. People like you show what's possible when passion is applied to purpose.

Adventure is out there

In 2019, Wendy Vitiritto and her family hiked the Grand Canyon rim to rim to raise funds for FA research and support services in honor of her 21-year-old son, Vinny. This year, Wendy decided to take on a new adventure and headed to Africa, where she climbed Mt. Kilimanjaro, the world's tallest freestanding mountain. Wendy hiked over 42 miles, traversed over 30,000 feet of elevation in eight days, and raised over $7,000! Wow! Wendy, we are so appreciative of your determination and drive to summit mountains in honor of individuals with FA around the world.

#FAmiliesAroundTheWorld unite during the month of May

During the month of May, we celebrate FA month, when families around the world share their stories, spread awareness, and raise funds for FA research and support services. This year, 656 donors gave 705 gifts to support 53 fundraising families from all over the globe. Together, they raised over $145,000 for FA research and support services! Thank you to every family, donor, and community member who raised funds, shared their story, and supported #FAmilyAroundTheWorld!

Record-breaking fundraising from Coley's Cause

The Levine family from Massachusetts hosted their 17th Coley's Cause fundraiser in memory of their daughter Nicole "Coley." Their community showed up for a wonderful day of golfing and raised a record breaking $38,000 for FA research and support services! Wow! Thank you so much, Todd and Kristin, for continuing to honor Coley's legacy through this fundraiser. Your community proves once again the power of friendship, legacy, and motivation to make a difference.

Team Bravery soars through national parks and fundraising goals

Team Bravery hasn't met a challenge they couldn't conquer. Earlier this year, FA parent and FARF board member Orion and the Team Bravery crew biked through six different national parks for a total distance of 460 miles. They were even joined by FARF's Executive Director, Mark Quinlan, as they completed yet another physical and fundraising feat. They raised over $95,000 for FA research and support services. Thank you, Team Bravery, for inspiring each of us to push ourselves to our limits to make change and honor those we love.

YOUR FA RESEARCH DOLLARS AT WORK

Research is the key to making Fanconi anemia (FA) a treatable condition so that those diagnosed with the disease can live long, healthy lives. That's why we've spent years supporting research that has identified the genes that cause FA, improved bone marrow transplantation, and uncovered the connection between FA and cancer. Thanks to this research, people with FA are now living longer and into adulthood. This milestone has now, unfortunately, presented people with FA with a new serious threat: cancer.

In recent years, much of FARF-funded research has focused on ways to prevent and detect cancer, and to develop less toxic therapies to improve and extend lives. As the research expanded, so did our approach to addressing this critical need. FARF has always supported basic science research projects and continues to do so, while at the same time seeking translational and clinical approaches. In addition to our research grant award program, we now have several research and clinical programs, including specialized focus-groups, a cancer clinical registry, a cancer early detection program, cancer consortium, and more. Below is a list of research and clinical programs you are supporting when you give or fundraise for FARF. These initiatives are possible thanks to the support of the whole community.

FARF Sparks Workshops

FARF Sparks is a workshop series designed to generate new ideas and maintain momentum for FA research in understudied topics. In these workshops, scientists share data and develop new research ideas that are focused on increasing understanding of FA and improving clinical care for individuals with the disease.

Upcoming workshops:

- Gene Editing
  In December 2021, FARF will host a workshop on FA gene editing. The goal of this workshop is for a team of researchers to pitch their ideas regarding a future FA gene editing grant that will be funded by FARF in 2022.
- FA-Associated Neurological Syndrome (FANS)
  FARF will host a workshop on Fanconi Anemia Associated Neurological Syndrome (FANS) in late October. This meeting will bring together families and clinicians to learn more about FANS and discuss potential prevention, detection, and treatment options.

Global Fanconi Anemia Brush Biopsy Program

The Fanconi Anemia Brush Biopsy (FABB) program is led by a group of researchers from the University of Düsseldorf and is focused on early detection of oral cancer in people with FA. The study has been funded by FARF and the German Support Group since 2016 and focuses on obtaining oral brush samples from FA patients to identify oral pre-malignant and malignant lesions. The study also provides educational materials on the importance of oral screening and the brush biopsy procedure for people with FA and their treating physicians. An expert panel of researchers, organized by FARF, meets quarterly to review FABB study milestone achievements and to cultivate global research partnerships.

Fanconi Anemia Cancer Consortium

The Fanconi Anemia Cancer Consortium consists of investigators from the NIH, Rockefeller University, University of Düsseldorf, and the University of British Columbia. This group aims to coordinate efforts on FA cancer research and clinical care that include clinical registry development, tissue repositories, and pre-clinical and clinical studies.

Fanconi Anemia Research Materials (the FARM)

The Fanconi Anemia Research Materials (FARM) repository facilitates FA research by providing materials to investigators at no cost. Materials that investigators can request include FA antibodies, human and mouse FA fibroblasts, and cancer cell lines. Learn more at https://www.fanconi.org/explore/research-materials.

FARF/Stand Up To Cancer Project

FARF joined Stand Up To Cancer® (SU2C) and three partner organizations to fund a $3.25 million dollar, three-year grant to a team of scientists who will focus on finding new treatments for FA and HPV-related head and neck cancers. The Research Team members were announced in Fall of 2021 and will begin their work in early 2022.

Joel Walker Cancer Ideas Lab

The Joel Walker Cancer series will bring in experts from various fields to focus on research and treatment specific to cancer in FA. Approximately 30 scientists will pitch their ideas in November to win $500,000 in grant funding from FARF.

Scientific Symposium

Every year, FARF gathers prominent and aspiring FA researchers and clinicians together to share the latest research and treatment updates and to form new collaborations. In July 2021, the Symposium was offered as a four-part virtual series on the topics of gene therapy, bone marrow

failure, DNA repair, and cancer. Because meetings occurred over Zoom, over 700 scientists from around the world were able to participate.

FA Patient Registry focused on FA Cancers

The FA Patient Registry is a critical way 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. The goal of the FA Patient Registry is to collect information about the natural history of FA cancers, including pre-cancer screening, diagnosis, treatment, and long-term follow up. The registry is patient-directed, meaning that individuals with FA and/or their families complete all surveys. The registry enables improved coordination with patient participation in FARF clinical programs such as the Virtual Tumor Board and the development of future therapeutic options. Although the focus of the registry is on FA cancers, enrolling prior to any cancer diagnosis should be a priority so clinical care can be expedited in the event of a diagnosis.

Virtual Tumor Board

The FARF Virtual Tumor Board (VTB) is a panel of physicians experienced with treating patients with Fanconi anemia (FA) who volunteer their time to discuss difficult FA solid tumor cases and offer treatment guidance. These volunteers are experts in hematology oncology, radiation oncology, medical oncology, and otolaryngology.

The VTB was developed to provide support to individuals with FA and their treating physicians, who may have less experience with treating cancer in individuals with FA. Members meet virtually with patients' treating physician(s) to review cases and provide input for treatment.

From September 2020 – October 2021, your contributions allowed FARF to invest $1,834,176 in research, including awarding the following projects:

Chemoprevention of Cancer in Fanconi Anemia

Investigator: Markus Grompe, MD

Institution: Oregon Health & Science University

Issue: Squamous cell carcinoma (SCC) of the oral cavity and anogenital area is very common in adult FA patients, and standard therapy for SCC is very difficult in these patients due to their hypersensitivity to DNA-damaging standard chemotherapy.

Project: This project will establish the best model for SCC screening and test the effects of a series of drug candidates.

Amount Funded: $97,067

Understanding Clonal Hematopoiesis in Fanconi Anemia to Improve Patient Surveillance Strategies

Investigator: Grant Rowe, MD, PhD

Institution: Boston Children's Hospital

Issue: Individuals with FA experience accelerated clonal hematopoiesis (CH), with onset as soon as the first few years of life. This results in a very high risk of FA patients developing myelodysplastic syndrome (MDS) and acute myeloid leukemia (AML).

Project: This project will introduce CH mutations found in FA patients into human progenitors to determine the effect of these mutations on HSC function and determine how CH mutations impact the DNA damage response machinery.

Amount Funded: $250,000

Acetaldehyde Exposure, Oral Microbiome, and DNA Damage in the Oral Cavity of FA Patients Before and After the Consumption of an Acetaldehyde-Rich Meal or a Low Alcohol Dose

Investigator: Silvia Balbo, PhD

Institution: University of Minnesota

Issue: Individuals with FA have a 500-700-fold higher risk of developing cancer compared to people without this disease, and acetaldehyde is believed to play a major role in the development of this cancer.

Project: The objective of this study is to quantify the increased sensitivity to acetaldehyde DNA damaging effects in FA patients by measuring acetaldehyde levels in the oral cavity of FA and non-FA volunteers before and after consuming a low dose of alcohol.

Amount Funded: $35,000

FARF is committed to supporting research to further treatments and a cure for Fanconi anemia. Over our 31-year history, we have funded 255 grants, 164 investigators, and 69 institutions worldwide. The total amount of research dollars awarded is $26,496,204!

FARF Leadership Updates

Thank you, Jack

Jack Timperley

Jack Timperley finishes term on FAdult Council

Jack Timperley joined the Fanconi Anemia Adult Council in 2019 as an inaugural member and served until July 2021. Jack brought both a contemplative and philosophical perspective to the FAdult Council by imploring the council and FARF to ask many questions to seek the most reasonable path towards advancing our mission.

Jack is a reflective and inquisitive person by nature, with a gentle and compassionate presence. He has an innate ability to make others feel comfortable and welcome around him. He brings a positive attitude to his work on the FAdult Council and within the rare disease community at large, including his recent work as a member of the Rare Action Network at the National Organization for Rare Disease (NORD), and his advocacy work with Be The Match.

To many, Jack is also known as Captain Marrow, who travels to hospitals, fundraisers, and conferences to give hope and guidance to patients and families undergoing medical procedures, while inspiring them to be their own heroes. It is for these reasons and many more that Jack was the proud recipient of the 2019 Amy Winn and Christopher T. Byrd award. We have a sincere gratitude and appreciation for all the time, energy, and work that Jack has given to the council. His presence will be greatly missed, but we are confident that his passionate commitment to this community will persist. You can follow Jack's ongoing advocacy work, listen to his podcast, and follow Captain Marrow on his website, www.jacktimperley.com. Thank you, Jack!

Welcome, new board members

Carmem Bonfim

Carmem Bonfim

Dr. Bonfim oversees the Pediatric Stem Cell Transplant Program at the Federal University of Paraná in Brazil. She brings to the board of directors many years of experience treating patients with Fanconi anema (FA) in Brazil, specializing in stem cell transplants. In addition to treating patients in Brazil, Dr. Bonfim serves as a point of contact for patients in other parts of Latin America and abroad. In 2016, she received the FARF Distinguished Service Award along with her colleague Ricardo Pasquini for her unwavering dedication and exemplary service to the FA community.

Pedro Ravelo

Pedro Ravelo

Pedro first learned about FA in 2001 when his then infant son Ivan was diagnosed. Over the last 20 years, Pedro and his wife Marina have connected with dozens of other FA families, participated in community events, and held several fundraisers. Pedro is the Director of Account Operations at Northwestern Memorial Hospital in Chicago, Ill., and has over 20 years of experience and extensive background in facilities trades, building automation, and mechanical systems.

Pedro brings to the board first-hand knowledge of the FA world, based on his personal experience as a father. He hopes that his personal struggles as a parent of someone with FA can inform the organization and provide support to affected families in the future.

If you or a loved one has received an FA diagnosis, please join the FA Patient Registry

WHO HAS JOINED THE REGISTRY?

145

people with FA have signed up for the registry (800+ receive this newsletter)

72

of these 145 are actively entering their information

DIDN'T I ALREADY REGISTER WITH FARF?

The FA Patient Registry is a patient-based study in which an adult with FA or the parents/guardians of a child/children with FA will consent and fill out questionnaires relating to the individual's medical and psychosocial experiences. The anonymous information gathered from this registry is then utilized by researchers to design research projects that will benefit the FA community.

Separate from the registry is the opportunity to enroll for our education and support services. Enrolling with FARF gives adults with FA and families of children with FA access to educational materials, resources, support services, and community. If you're receiving this newsletter you are enrolled in FARF Family Services and we now encourage you so sign up for the FA Patient Registry.

WHAT NEEDS TO HAPPEN NEXT

Getting registered is the first step, then entering your data is **vital** to providing the critical information that clinicians and researchers need to truly make a difference. More than 1,500 patients are enrolled in FARF family services. The goal is that each one of you is also signed up with the FA Patient Registry. Taking on cancer and FA takes all of us.

HOW TO JOIN THE REGISTRY

Visit fanconiregistry.iamrare.org to create your private account

Answer questions about your personal info and experience with FA. Come back to complete at any time.

Know that you're helping researchers find answers faster

Fanconi anemia is a rare disease and can be hard to diagnose and treat. Research is the key. And the key to research is you.

If you have been diagnosed with Fanconi anemia, or your child has FA, join the registry today.

JOIN THE FA REGISTRY TODAY: HTTPS://FANCONIREGISTRY.IAMRARE.ORG

Welcome, new members of the FARF team!

Laura Hefner
Research Program Coordinator

Originally from the suburbs of Chicago, Laura graduated from Northwestern University, where she earned a degree in Social Policy and French. Following graduation, she worked at the American Society of Nephrology in Washington, D.C. for two years. Laura recently earned her Master of Public Health degree from Emory University. During her time at Emory, Laura served as the treasurer for the Emory chapter of the Georgia Public Health Association and was a graduate research assistant for the Global Fortification Data Exchange.

At FARF, Laura leads the administration of the research grants process, develops programs for scientific events, and helps to communicate FARF's scientific impact to stakeholders. She is looking forward to working alongside researchers committed to finding a cure for FA. After having lived in the Midwest, Northeast, and South, Laura is excited to be in the Pacific Northwest at FARF headquarters in Eugene, Oregon. In her free time, she enjoys exercising, reading, and spending time outdoors.

Andrea Ronan
Clinical Science Program Manager

Andrea comes to FARF with 10 years of healthcare experience specializing in nutrition support and critical care. Originally from Pennsylvania, she most recently worked at Walter Reed Military Medical Hospital in Maryland before relocating to the Pacific Northwest. Andrea is a registered dietitian and will bring this knowledge and experience to her role as Clinical Science Program Manager at FARF. She manages the FA Patient Registry, oversees the process of updating the FA Clinical Care Guidelines manual, and leads the Virtual Tumor Board to make sure individuals with FA who have a cancer diagnosis get the best possible care. "I'm excited to help make a positive impact by continuing to grow the science programs at FARF while working closely with the FA community," Andrea says.

A die-hard Penn State football fan, when she's not working you can catch her rooting for her team, spending time with her American Bulldog, Boh, baking, or traveling.

Rosie Holcomb
Family Services Program Manager

Rosie grew up in Central Oregon before moving west to attend the University of Oregon, where she majored in biology with minors in sociology and chemistry. Before joining the FARF team, her interest in nonprofits led her to work for the 4-H program in Central Oregon, then at United Way of Lane County, where she developed new programs and relationships with members and donors. In her role as Family Services Program Manager at FARF, Rosie assists caregivers and individuals affected by FA by organizing meaningful events, creating support and educational resources, and providing direct support along the FA journey. "I'm extremely thankful and excited for the opportunity to make a difference in this role that perfectly connects my love of science and helping others," Rosie says.

Outside of work, you can find Rosie reading a historical fiction or mystery novel, biking next to the river, eating at her favorite local Thai restaurant, or spending time with friends and family.

Jess Stafford
Gift Processing Coordinator

Born and raised in Eugene, Jess brings experience and a passion for nonprofit work to the FA Research Fund. Before joining the FARF team, she spent years volunteering and working in nonprofits, most recently at Volunteers in Medicine in Lane County. "I'm driven by work that allows me to see real-life impact in the communities I serve." At FARF, Jess assists with fundraising and administration. She is the smiling face behind the scenes who processes donations, issues tax receipts, and is the point of contact for all donation questions.

A true Oregonian, Jess loves exploring the outdoors with her family. She's a big track and field fan and is a proud comic book nerd.

5th Edition of the Clinical Care Guidelines now available in Spanish AND online

The newest edition of the Fanconi Anemia Clinical Care Guidelines (fifth edition) is now available in Spanish and in English online and in print. You can download it free on the FARF website or request a copy for yourself and your doctor.

When you registered with FARF Family Services, you received a handbook about managing Fanconi anemia. This was likely the 4th edition, published in 2014, or maybe one of the previous editions.

The fifth edition is a revision of the fourth edition. The contributing authors are physicians or clinical care providers with expertise in treating patients with FA. The fifth edition provides evidence- based recommendations and is geared toward clinical providers as the primary intended audience. Patients and families who wish to secure optimal treatment by improving their understanding of FA may also benefit from this edition.

The *Guidelines* start with a brief summary of the molecular mechanisms of the FA DNA repair pathway and the diagnostic testing process for FA. Subsequent chapters examine more specific health issues faced by people with FA, including hematologic issues, squamous cell carcinoma, oral and dental care, gynecologic care, dermatologic care, gastrointestinal issues, endocrine disorders, hearing and ear issues, and skeletal abnormalities. Finally, the guidelines conclude with a summary of clinical care recommendations for patients with FA.

Download, view, or request a hard copy at **www.fanconi.org/explore/clinical-care-guidelines**

FAMILY FUNDRAISING LIST

From January through August 2021, FA families have raised more than $734,000 for the Fanconi Anemia Research Fund! 196 families raised funds with 94 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.

$100,000+

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

$50,000 - $99,999

Orion and Lisa Marx

$20,000 - $49,999

Coley's Cause Foundation (Todd and Kristin Levine)
John and Kim Connelly
Alan and Rachel Grossman

$10,000 - $19,999

Ryan and Rebecca Brinkmann
Mauro and Kerrie Cazzari
Edward and Janice Duffy
Kevin and Lorraine McQueen
Ian and Tricia Mitchell
Mark and Diane Pearl
Michael and Beth Vangel

$5,000 - $9,999

Rachael Alaniz and Kevin Gatzlaff
James and Jennifer Armentrout
Sean and Allison Breininger
David and Kim Chew
Andre Hessels and Rutger Boerema
Charles and Kathleen Hull
Andrea and Robert Sacks
Callie Toal and Julie Leeds
Joe and Wendy Vitiritto

$1,000 - $4,999

Brian and Carly Adel
Adam and Marissa Becker
David and Sarah Borden
Chris and Jennifer Branov
Andrew Coons and Valeen Gonzales
James and Crystal Eubank
Scott and Windy Farmer
Britteny Ferrin
David and Mary Ann Fiaschetti
Emmanuel and Dana Gallegos
Sindhuja Gangadurai
Stanley Gilbert
Andrew and Jennifer Gough
Gary and Heidi Grassi
Rachel and Zachary Gratz-Lazarus
Owen Hall and Margaret Kasting
John and Martina Hartmann
Barbara and Chris Hawkshaw
Helen and Sean Healey
Steven Holmes and Kerry Robinson
Brian Horrigan and Amy Levine
Shawn Huff
Jeff and Beth Janock
Craig, Rebecca, Brett and Michael Johnson
Stan and Michelle Kalemba
Stephen and Jennifer Klimkiewicz
Kristine la Cour Rasmussen
Timothy and Mary Ann Lana
Robert and Anna Langtry
Todd and Kristin Levine
Michael London
James and Holly Mirenda
Tyler Morrison and Rachel Altmann
Nancy Nunes
David and Stacy Ownby
Peggy Padden
Chris and Mel Payne
Peter and Janice Pless
Mark Ritchie and Lisa Mingo
Emily Salo and Kenn Lonnquist
Colleen Scholl
Kelly Semkiw
Bradley and Darlene Starner
Janice and Kenneth Sysak
William and Mary Underriner
Gerard and Cynthia Vandermeys
Nigel and Ann Walker
Anthony Walsh
Jessica and Ezekiel Werden

Up to $999

Virginia Abello, MD
Peter and Donna Abramov
Michael and Jennifer Aggabao
Assila Al-Marshoudi
Marzban and Daisy Ardeshir
Charles Balow and Xandra Towndrow
Faith Barbe and Shane Estelle
Amanda Barber
John and Audrey Barrow
Mark and Linda Baumiller
Israel and Mary Jo Becerra
Domenico Bertolucci and Federica Bonati
Randolph and Nancy Bloxom
Richard and Tena Boson
David Boudreaux
Sean Bowen
Jason Brannock
Michael and Tracy Brannock
Donald and Danielle Burkin
Jackie Burton
Robert and Barbara Capone
Michael Cavanaugh
Amy Chadburn
Mary Eilleen Cleary and Gleaves Whitney
Stuart Cohen and Deane Marchbein
Bill and Pat Danks
Richard Day
Larry Davis
Darrel and Kalani DeHaan
Jeremy and Michelle DellaValle
Brian and Jennifer Dorman
Delbert and Linda Dotson
Lindsay and Sandra Dunn
Oscar Duque and Yanira Ramirez
Curt and Crystal Fales
Carole Felmy
Brian and Cindy Fuller
Melody Ganz
G. Mitzi Gerber
Madeline and Patrick Gregg
Benjamin and Stephanie Griggs
David Guidara
Florence Guidinger
Shawn and Doreen Gummoe
Rachel and Kristian Guttulsrud
Gary Haftek
Paige Halverson and Tom Fairbanks
Eric and Elisabeth Haroldsen
Robert and Victoria Hathcock
Nora Herren
Lisa Jansen van Rensburg
Mary-Beth and Ben Johnson
Ashleigh Kamsickas
Angela Keaton
John and Karilyn Kelson
Dan Klug and Elizabeth Bertrandt-Klug
Daniel and Aeileeng Kold
Verena Konig
Christopher and Dana Lamb
Peg LeRoux
Tanner and Jessica Lindsay
Keith and Jessica Loo
Col Gregory & Lt Col Lynnette Lowrimore
William and Jacquelyn Lucarell
Kristina Mack
Esther and Jamison McKellar
Daniel and Angie McMahon
Adam and Olivia Mindle
Kelly and Gerald Mlachak
Kate Montgomery
John and Betty Mozisek
Kenny and Lisa Myhan
Tony and Lina Nahas
Louis and Virginia Napoles
Lisa and Jack Nash
Philip Nelson and Candy Lindsey
David and Catherine Neumann
Shawna and James Nicholson
Robert and Mary Nori
Eliana and Adriano Ortiz
William, Kelli and Kit Owen
Seth Parelman
Melissa, Dave and Axel Perdue
Tim and Ashleigh Pinion
Lynn and Shirley Quilici
Senthilkumar Radhakrishnan
Kelsey Robinson
Neil and Emily Robison
Maria Rocha and Gabriel Gallegos
Kevin and Katherine Rogers
Les and Nancy Ross
Craig and Alisha Rushing
Richard and Marilyn Sablosky
Jennifer and Brian Sadlowe
Colleen Satterlee
Ron and Alice Schaefer
Zoey and Noah Scheider
William and Connie Schenone
David and Karen Sheppard
Bryan and Karen Siebenthal
Jamie Sileo
Julia, Wilhelm and Lorelei Skakandy
Jamie Slappo
Karin Staab
Lea Ann and Jeff Stiller
Sharla and Josh Strickland
Charles and Jennifer Sumrall
Sharon Swanson
Ana Alejandra Tabar Concha and Elvin Estevez Lopez
Mary Tanner
Holly and Iain Taylor
Mark and Susan Trager
Joseph and Natalie Vitrano
Herb and Jeanette Webb
Marc Weiner
David and Erica Williams
Michael and Kimberly Williams
Alex Winn
Sacha Wizman
Chad and Dawn Wood
Kyle and Madison Wright
Wesley and Susan Wycoff
Jian Yang and Jing Nie
Sean and Kristin Young

Season of Promise, *continued from page 3*

https://fundraise.fanconi.org/holidays

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

How has another year already come and gone? In the blink of an eye, the holiday season is upon us again. This year, (insert short personal update about what your family has been up to in the past year).

As you know, another part of our story is that __ has 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. Each year at this time, we write to our friends and family to fundraise for the Fanconi Anemia Research Fund (FARF).

We do it because FARF feels like an extension of our family. Because they fund research for better treatments and a cure for Fanconi anemia. Because they provide support services to families like ours around the world.

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 help FA families navigate the FA diagnosis, connect with other FA families around the world, and feel hope for the future.*
- *You invest in better outcomes for everyone touched by cancer. By funding FA research, you're helping to unlock the mysteries of DNA repair problems that impact all of us. This year, FARF partnered with Stand Up To Cancer to fund the biggest project yet addressing cancer in people with FA.*
- *You support research in gene therapy, gene editing, and advances in bone marrow transplants.*
- *You fund clinical trials to advance therapies for kids and adults with Fanconi anemia.*

We humbly 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 helping us spread hope and love in the community, especially this year. We wish your family a [peaceful/restful/restorative] holiday season!

The _____ Family

DONATE WHILE YOU SHOP ON AMAZON

We are all shopping more online these days. When you buy on Amazon, you can designate FARF as your charity of choice, and we will receive a portion of the sales. Visit smile.amazon.com, select Fanconi Anemia Research Fund as your charity, and start shopping. That's it!

FANCONI ANEMIA RESEARCH FUND
Advancing Research. Supporting Families.

360 E. 10th Ave, Suite 201
Eugene, Oregon 97401

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)

Donations by Phone:
Call us at 541-687-4658 or toll free at 888-FANCONI (USA only)

Donations by Mail:
360 E. 10th Ave., Suite 201, Eugene, OR 97401

Donate While Shopping on Amazon:
www.smile.amazon.com. Choose Fanconi Anemia Research Fund.

Donations of Appreciated Stock:
Please contact our office at 541-687-4658 or email info@fanconi.org.

fax: 541-687-0548 • info@fanconi.org • www.fanconi.org

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.*