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FA Family Newsletter

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Unforgettable Memories Made at Annual FA Family Meeting



“Camp is priceless! The parents and children get support, hope, love, encouragement, knowledge, and a real sense of friendship that knows no boundaries.” –Camp attendee

The Fanconi Anemia Research Fund’s 24th annual FA Family Meeting brought together 53 families from five countries for a week of festivities, bonding activities, research opportunities and informative sessions. Fifty-seven adults and children with FA attended Camp Sunshine in Casco, Maine, with siblings, parents and other family members. The addition of an extra day to this year’s meeting allowed first-time families to arrive early and get acquainted with the campus and the process as well as to connect with each other.

Nineteen speakers shared their insights in a series of thoughtful sessions, including updates on current research, a fundraising seminar, a panel of FA parents sharing their experiences with bone marrow transplants, and strategies to cope with stress. Medical and educational sessions covered topics pertaining to the treatment and management of FA, notably the risks of oral

cancer, the use of androgens, and the effects and follow-up of stem cell transplantation. Articles covering several of these presentations can be found inside this issue.

Support sessions with Nancy Cincotta, Psychosocial Director of Camp Sunshine, addressed some of the issues faced by FA individuals, their parents, and siblings. “These meetings are unbelievably special.

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We Want Your Opinion

The Fanconi Anemia Research Fund FA Family Newsletter is ready for a makeover! We at the Fund strive to make the newsletter an excellent resource with timely and interesting information related to Fanconi anemia and those affected by FA.

We ask all readers to take 5-10 minutes to fill out a brief survey online. This will help us improve the content, form, and layout of the newsletter.

Thanks! We greatly appreciate your time and opinions.

To access the survey, visit

http://fanconi.org/index.php/publications/family_newsletter_survey

To receive a hard copy or for any questions, email sherri@fanconi.org.

Unforgettable Memories Made at Annual FA Family Meeting

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So much love and support come from them! None of us is in this alone,” one participant shared.

Some of the most memorable moments were the fun-driven social activities like the adult banquet and karaoke night, bass fishing on the lake for the kids, a night swim in the indoor pool, and a dance party for adults with FA. Another highlight was the outdoor barbecue, where families gathered to share a meal under a beautiful wooden structure built by Jeff Boggs.



Camp attendees participated in a truly unique experience by releasing butterflies in memory of Fund co-founder Dave Frohnmyer. A beautiful bench crafted by the Boggs Family was also dedicated in memory of Dave in the Fanconi Garden near the lake (see page 13).

Thanks to the devotion of the speakers, staff, volunteers, and of course, the families, this year’s FA Family Meeting inspired, educated and encouraged all participants. We look forward to seeing you again next year!

Gene Therapy Phase 1 Trial in Seattle Seeks Participants



Jennifer Adair, PhD, Fred Hutchinson Cancer Research Center

Dr. Jennifer Adair is looking for patients to take part in a Phase 1 gene therapy trial in Seattle, in which researchers will put a functional *FANCA* gene into a patient’s bone marrow stem cells outside the body, then reintroduce those cells. This technique has proven safe and successful in gene therapy for other genetic diseases. So far only two Fanconi anemia

patients have been treated. From them we have learned that very few stem cells are present in FA bone marrow, but that these cells can be kept alive outside the body, with successful gene transfer in under 24 hours. Afterwards, small numbers of gene-modified cells can be detected in the blood and bone marrow. Blood cell counts in these two recipients have returned to better than, or equal to, baseline, pre-gene-therapy

levels. The Seattle trial hopes to find out whether enough stem cells can be collected to really make a difference and whether this difference would constitute a short-term bridge therapy on the way to transplant, or whether benefits could last a lifetime.

To be eligible to take part in this trial, you must:

- Be 4 years of age or older;
- Have near-normal function in kidneys, liver, and lungs;
- Have near-normal bone marrow cytogenetics and adequate blood counts;
- Be infection-free and free of any cancer with a survival rate under two years;
- Be free of heart disease, hemophilia, HIV, or uncontrolled diabetes;
- Not have a matched sibling donor;
- Not be pregnant.

The protocol calls for you to spend seven to eight weeks in Seattle, with monitoring afterwards at home. All expenses of the trial are covered by the Cancer Center. Travel and lodging in Seattle are not covered, but the Fanconi Anemia Research Fund can provide scholarship assistance.

What's New in FA Research, Trials and Treatments?



Richard Gelinas, PhD, Institute for Systems Biology, Scientific Advisory Board and Board of Directors, Fanconi Anemia Research Fund

In an exciting and hopeful presentation, Dr. Gelinas shared information about new drugs to address bone marrow failure and head and neck squamous cell carcinoma (HNSCC), as well as a new way to screen for potentially beneficial drugs.

Several labs are pursuing research on Fanconi anemia drugs. Three labs are examining different ways to relieve stress

from DNA damage and the poor recycling of **aldehydes**. Metabolism naturally produces aldehydes inside of cells. If they aren't recycled promptly, however, DNA damage can result, which is particularly problematic for those with FA.

- Dr. Michael Garbati and Dr. Grover Bagby (Oregon Health & Science University) are exploring whether a small molecule drug can enhance aldehyde removal. They are partnering with the company Aldea Pharmaceuticals, which has been working on the poor recycling of aldehydes in Asian Flushing Syndrome. Together they will examine the use of this small molecule drug to improve aldehyde recycling in FA. Dr. Ray Monnat (University of Washington) is testing whether aldehyde scavengers protect stem cells from damage or killing by aldehydes *in vitro* and *in vivo*. And Dr. Markus Grompe (OHSU) is testing an approved aldehyde scavenger drug in FA mice.
- One promising new drug is **N-acetyl cysteine (NAC)**. NAC inhibits cellular damage induced by oxygen. The hope is that NAC will improve blood cell counts and reduce DNA damage. It dissolves in liquids so is easy to take, has been used successfully with some other diseases, and is approved for use in children. A multi-center trial to enroll 40 FA patients should be starting soon. Participating centers are in Canada, Italy and the United States. The study coordinator, Dr. Yigal Dror, expects the trial to enroll subjects late this year. To learn more, visit www.sickkids.ca/AboutSickKids/Directory/People/D/Yigal-Dror.html.

- Jordi Surrallés (University of Barcelona) is investigating a **new method to screen approved drugs**, which should help pinpoint which drugs are most likely to be beneficial. In a very new study, Susanne Wells (Cincinnati Children's Hospital) is testing another class of drugs called PARP inhibitors as a therapy against HNSCC.
- HNSCC is the sixth most common cancer worldwide, with over half a million new cases each year, but the incidence in FA is about 700 times that of the general population. Those with FA have limited treatment options due to their sensitivity to chemotherapies and radiation. FARE is funding a study by Dr. Agata Smogorzewska (The Rockefeller University) to examine the DNA of FA SCCs for drug-responsive mutations. Dr. Smogorzewska will also collaborate with other scientists, like Dr. Wells, to test multiple drugs simultaneously.
- The National Cancer Institute (NCI) is also studying HNSCC. The Cancer Genome Atlas is asking questions such as: Why do the cancers develop? Which genes have mutations? What is the role of papilloma virus? Why are they so hard to cure? SCCs from 528 (non-FA) patients were analyzed using multiple methods. Knowing the gene defects in a specific SCC may lead to more targeted therapies, or "precision medicine." NCI will soon be opening a large-scale trial for adults called **NCI-MATCH**. They will analyze a patient's tumor for genetic abnormalities, and determine if the abnormalities match a mutation targeted by a drug being used in the trial. This is a very exciting frontier of cancer research and treatment.
- Another new approach in cancer research is called **immunotherapy**. Tumors tend to be hard to kill because they look normal to the immune system, yet they can also suppress the entire immune system. Several big pharmaceutical companies are developing drugs to address the T-cell activation issue. While current studies are in the non-FA population, Dr. Gelinas thinks immunotherapy has potential for FA as well.

Dr. Gelinas concluded that "better informed patients take better care of themselves." The newsletter and handbook are invaluable sources of information. This couldn't be a more powerful reminder of how important it is to fundraise! Every donation makes this research possible, and gives all FA families hope for the future.

Stem Cell Transplantation: Updates and Challenges



Margaret MacMillan, MD, University of Minnesota; Parinda Mehta, MD, Cincinnati Children's Hospital Medical Center; Michelle Lee, MD, Boston Children's Hospital.

The prognosis looks very good for low-risk FA children, but transplant outcomes for FA adults are much poorer. The challenge for adults is to monitor their health and blood counts through regular check-ups and stay abreast of medical and transplantation research, so that if they need a transplant they can act quickly. This was the update provided at Camp Sunshine by experts from three different transplant centers in the US in a talk about stem cell transplant outcomes and future directions at their centers.

Two researchers, Michelle Lee and Parinda Mehta, discussed results from a seven-year multi-institutional study at five transplant centers in the US. Results were presented last summer at Camp Sunshine and summarized in the Fall 2014 Family Newsletter (p. 11). This project involved 45 FA children and adults, all of whom underwent a radiation-

The clear message to all FA adults is to get regular checkups and pay attention to the latest medical developments, in order to increase their chances of survival.

free transplant preparation process. Overall survival is 80%. Low-risk children—those under 10 years of age, without myelodysplastic syndrome (MDS) or leukemia, and transplanted for progressive marrow failure—had the most promising results. One hundred percent survived!

According to Dr. Lee, stem cell transplantation procedures continue to improve dramatically. The question now is how far doctors can go to reduce the toxicity of pre-transplant

conditioning, especially in lower-risk patients. Potential advantages include decreased need for pain killers and decreased risk of secondary infections. Potential concerns include the possibility of graft failure, as well as failure to eradicate pre-malignant clones.

Dr. Mehta focused on improved outcomes in unrelated donor transplants, especially for children with non-malignant disorders. According to her, transplantation results for young people with FA are now excellent, but we need to make progress for older patients and those with MDS/leukemia. She gave an update on the current open study at Cincinnati Children's Hospital entitled 'Risk-adjusted chemotherapy only preparative regimen (NO radiation) for FA.' In that study, busulfan chemotherapy doses are adjusted according to patients' ages and conditions. Eight patients were treated in this new protocol, and they all engrafted. Seven are alive and doing well.

A third speaker, Margaret MacMillan, MD, updated attendees on transplant outcomes from the University of Minnesota Children's Hospital, which uses a low-dose radiation transplant protocol. Survival at five years after transplant for low-risk patients with no transfusions or serious infections is now 94%, but increased age, a history of one or more transfusions, and life-threatening infections greatly decrease survival. Only three of seven adults survived transplant. Another high-risk group is *BRCA2* patients. Of 10 *BRCA2* patients transplanted at Minnesota, only three survive, for two, eight and 10 years, respectively.

Dr. MacMillan emphasized the uniqueness of every FA patient. She said, "We are doing much better than we were ten years ago," but age, infections, and transfusions increase mortality risk. The challenges presented by age are especially important, in that healing becomes more difficult for everyone as they age. FA children, once diagnosed, are carefully monitored by parents and physicians, but FA adults who are not symptomatic sometimes fail to get yearly checkups and wait too long to seek treatment. By the time they reach transplant, too often a health crisis has developed. Increased risk factors include even one transfusion, serious infections, or chronically low blood counts. These risk factors go far toward explaining the low adult survival rate in both the Minnesota study and the multi-institution study. The clear message to all FA adults is to get regular checkups and pay attention to the latest medical developments, in order to increase their chances of survival.

Using Androgens to Stabilize Blood Counts in FA



Eunike Velleuer, MD, Children's Hospital, University of Dusseldorf, Germany

Androgens are highly effective in improving blood counts in the majority of Fanconi anemia patients and can have long-term beneficial hematological effects without excessive toxicity, according to Dr. Eunike Velleuer. Newer androgens such as danazol are far less masculinizing than oxymetholone, are less toxic, and can be effective. Dr.

Velleuer discussed the pros and cons of androgen use in this stimulating lecture.

Over the years, expert opinion based on anecdotal accounts was that approximately 50% of FA patients experienced a transient hematological response to androgens but that long-term responses were rare. Dr. Velleuer believes this pessimistic conclusion is not accurate.

At our 2014 Scientific Symposium, Lisandro Ribeiro, MD, Federal University of Parana, Brazil, Bone Marrow Transplant Unit, presented a study of 49 FA patients who underwent oxymetholone therapy between 2005 and 2013. Forty FA patients (82.6%) showed hematological response and were no longer transfusion dependent at a median of three months after beginning therapy, and 50% had a response in all three blood lineages.

The advantage of androgen therapy is the extremely low risk of short or long-term therapy-related mortality. Bone

marrow transplant, while the only cure for bone marrow complications, is still NOT a low-risk therapy. We have a long history of experience with androgen use, and the side effects, which are related to dose and type of androgen used, are well documented. They include virilization (acne, facial hair growth, deepening of the voice, development of pubic hair and enlargement of the penis/clitoris), growth spurt in young patients followed by premature closure of the epiphyses (resulting in shorter adult height), and hyperactivity and behavioral changes such as aggressiveness. Cases cited in the literature 13 years ago describe liver adenomas (benign tumors) and, rarely, liver cancer in FA

Dr. Velleuer stated that androgens do not negatively affect transplant results. However, androgens don't prevent myelodysplasia or leukemia, so patients may later need a transplant. They will be older, and increased age STILL has a negative impact on transplant outcomes.

patients treated with androgens. Peliosis hepatis, a vascular condition characterized by multiple blood-filled cavities throughout the liver, is cited as a rare complication. Danazol and lower doses of androgens may have eliminated or greatly reduced risks to the liver. Dr. Velleuer noted that during the last 15 years, no liver cancer has been detected in German patients taking androgens.

Dr. Velleuer stated that androgens do not negatively affect transplant results. However, androgens don't prevent myelodysplasia or leukemia, so patients may later need a transplant. They will be older, and increased age STILL has a negative impact on transplant outcomes.



FA Guidelines for Diagnosis and Management

The Fanconi Anemia Research Fund is pleased to announce the publication of *Fanconi Anemia: Guidelines for Diagnosis and Management, Fourth Edition*. Published in 2014, the updated guide replaces the third edition.

Every family registered with the Fund has been sent a printed copy of the new *Guidelines*. If you would like additional copies, please use the link on the Guidelines page of our website or contact the Fund. The complete electronic version is also available on the Fund's website.

Overview of FA Cancer Risk and Future Therapies



Carter Van Waes, MD, PhD, Chief, Head and Neck Surgery Branch and Clinical Director, NIDCD

Dr. Van Waes discussed the risk factors, prevention strategies and future therapies for Fanconi anemia patients at high risk for head and neck squamous cell carcinoma (SCC). SCCs are the cancers arising from the flat cells that line our oral cavity and throat.

Everyday injuries such as biting, poorly fitting dentures and burns from very hot

foods and liquids stimulate division of these cells. While dividing, they copy their DNA, a process which is more susceptible to damage. People with FA are unable to repair damage to DNA. Chemicals made by our bodies as part

The emerging focus on new precision medicines that target specific genetic mutations holds special promise for individuals with FA.

of normal cellular metabolism, and by bacteria in our mouth can damage DNA. Chemicals from tobacco and alcohol are especially toxic. The human papilloma virus

(HPV) can infect cells and insert viral genes into DNA that are implicated in cancers of the tonsil and a subset of FA gynecologic cancers.

Reducing these hazards is key to prevention. Dr. Van Waes was adamant that **FA patients must avoid tobacco and alcohol**. Males and females should be immunized against HPV beginning age 11-12, as currently recommended by the American Association of Pediatrics. Patients should reduce injury to cells in the oral cavity (don't consume very hot foods and liquids; avoid oral cavity trauma), and minimize dental x-rays and CT scans.

The emerging focus on new precision medicines that target specific genetic mutations holds special promise for individuals with FA. New therapies are being tested based on the discovery that some tumors respond to specific drugs or immune therapies that can target some genetic mutations. FA SCCs may have chromosomal genetic mutations typically found in non-FA SCCs, suggesting that therapies that are effective in the general population might help FA patients as well. Dr. Van Waes listed several drugs that have generated a great deal of excitement and might be appropriate for inclusion in persons with FA cancers in clinical trials, including metformin and immunotherapy.

Some cancer centers are beginning to sequence the DNA of tumors for genetic mutations and are searching for compounds that target cancer-causing mutations. FARE is currently funding studies to identify the genetic mutations in HNSCC from FA patients. In the future, this approach will be widely available, and could add a desperately needed therapy for FA cancers.



Endocrine Update

The takeaway from Dr. Susan Rose's presentation this year is that **ongoing discoveries continue to clarify the roles of various genetic mutations in causing growth deficiencies**. Meanwhile, it is important to involve an endocrinologist early on to track stature, glucose, puberty, and bone density in everyone with FA. Dr. Rose also stressed the general health benefits of calcium, vitamin D, exercise, and a low-glycemic diet. Although understanding the genetic factors of FA are a challenge, **good medical care and smart lifestyle choices can strengthen the health of all people with FA**. For more information, see a summary of Dr. Rose's 2014 presentation about key endocrine issues for FA in the 2014 Fall Family Newsletter (page 6).

German Study of Oral Cancer Risks



Ralf Dietrich, FA parent, German Fanconi Anemia Patients Organization and Research Fund; Eunike Velleuer, MD, Children's Hospital, University of Dusseldorf, Germany.

The Spring 2015 FA Family Newsletter presented results from the 2013-2014 German oral cancer screening study of 300 individuals with FA, comparing incidence of oral lesions and oral cancers between transplanted and non-transplanted patients. Eunike Velleuer and Ralf Dietrich concluded that transplanted patients have twice as many oral lesions and nearly twice as many cancers as those who have not undergone transplant. Patients who develop cancer following a transplant are generally younger than those who have not undergone transplant.

At our 2015 FA Family Meeting, Velleuer and Dietrich elaborated on their findings:

- Every diagnosed oral cancer started with a visible lesion.
- **Most visible lesions are not malignant.** Of 106 visible lesions from both the transplanted and non-transplanted groups, 13 were diagnosed as cancer.
- Prior to bone marrow transplantation, males have a much higher incidence of oral lesions than females. Dr. Velleuer attributes this difference to lifestyle: her patient interviews showed that males are far more likely to smoke and drink than females. After transplant, the high risk of oral lesions becomes the same in males and females.

- Dr. Velleuer noted that cancers of the gingiva are quite frequent and appear more aggressive than other oral cancers. Six of the 13 oral cavity cancers were localized to the gingiva. **Insist that your physician screen this area very carefully!**
- Four of the ten transplanted patients diagnosed with cancer were young (ages 17.2-20.5). The three non-transplanted patients were 30.5, 31.1 and 40.3 years of age at the time of cancer diagnosis.
- The specific type of conditioning (non-radiation versus radiation) did not appear to affect the incidence of oral cancer in the transplanted group. Dr. Velleuer speculated that the prolonged time without a well-functioning immune system, rather than the type of conditioning used prior to transplant, might account for the increased incidence of cancer in transplanted patients.
- Graft-versus-host disease (GvHD) was diagnosed in seven of ten transplanted patients who developed oral cancer. Five patients had acute GvHD, one had chronic GvHD, and one patient experienced both.
- Cancer is the primary cause of death of adults in the

Transplanted patients have twice as many oral lesions and nearly twice as many cancers as those who have not undergone transplant. Patients who develop cancer following a transplant are generally younger than those who have not undergone transplant.

German support group. The Germany study followed 23 adults, ages 21-52. Thirteen in this group have died, ten due to squamous cell carcinoma.

Dr. Velleuer suggested the need for regular self-exams in an effort to detect early signs of lesions. The Fanconi Anemia Research Fund will develop and post on our website a short video demonstrating self-examination techniques.

Oral Cancer Fact Sheets Available

Regular screenings for oral cancer are critically important for people with FA. The Fund has fact sheets about squamous cell carcinoma to share with your dentist and ear, nose and throat doctor (ENT). **FA patients and families are encouraged to take a fact sheet to every dentist and ENT visit.** The fact sheets—in English, Spanish, Afrikaans, Dutch, French, German, Hebrew, and Italian—are available on our website or by calling our office.

Families Tell About Transplants

At Family Camp, three FA moms shared their stories of the bone marrow transplant process. Mary Ann Fiaschetti, whose son Peter was successfully transplanted at age 10, stressed the importance of learning as much as possible in advance, attending Family Camp, seeking treatment at a recognized transplant center, establishing a good relationship between your local hematologist and FA experts, adopting healthy habits, raising money for FA research, and helping your child enjoy holidays and special treats despite the need for isolation. According to Mary Ann, you should request a case manager from your insurance company, then fight hard if you need an out-of-network provider. One of her most practical hints involved making many copies of a one-page (two-sided) medical history that also includes contact information for doctors and insurance carriers, and carrying it with you at all times. At home, be honest with your child in an age-appropriate way and discuss the transplant process well in advance. At transplant time, personalize the room with family photos and favorite things. The Hospital Education Coordinator can establish a video link with your

These three families are living proof that although the transplant experience is no walk in the park, chances of success are better than ever, and along the way there are experiences and friendships that can inspire us for life.

child's classroom. Accept help from friends and family! Going through the transplant process together can make a family stronger.

Mary Ann Lana, whose son Eli was successfully transplanted in 2011 at age 6, had to cope with her own

sense of helplessness and life out-of-control, as well as her son's steroid meltdowns, mucositis flare-ups, complications with viruses, black tongue, isolation and germ phobia. The biggest challenge was to treat him like a normal kid. She did that so well that he wound up writing a book about his experience, "Going to the Hospital." It's filled with great advice about how to treat a doctor ("Don't fool around!"); what to play with (stuffed animals, coloring, puzzles, board games, iPhone apps, Youtube videos); what to eat (snacks, chocolate milk!); and how to score treats and gifts ("If you do really good, you should get a present.").

Marina Ravelo, mother of Ivan, now eight years post-transplant, was fortunate to be able to give birth by preimplantation genetic diagnosis (PGD) to son Fabian in 2005, whose sibling cord blood could be hand-delivered to the University of Minnesota, where it would be stored until Ivan's transplant in 2007. The family stayed at the Ronald McDonald House, where there was terrific support—meals, school for siblings, close proximity to the hospital—as well as challenges: a long wait list, and then the germs of 48 other families. Ivan was a "rock star" at the hospital, discharged after only 17 days. Though he soon developed a common virus that created an irritation to the lining of his bladder that lasted 6 months, he never had to be readmitted to the hospital. He was able to return to school in 2008. Right now Ivan does not take any medications as he prepares to enter high school in the fall of 2015.

These three families are living proof that although the transplant experience is no walk in the park, chances of success are better than ever, and along the way there are experiences and friendships that can inspire us for life.



Mary Ann Fiaschetti



Eli, Tim and Mary Ann Lana



Ivan Ravelo

Stem Cell Transplantation: Long-Term Effects and Recommended Follow-Up



Margaret MacMillan, MD, University of Minnesota

Dr. Margaret MacMillan discussed important long-term effects of living with Fanconi anemia after a stem-cell transplant. She also addressed some issues that are important whether a person has been through transplant or not. She stressed the importance of close monitoring and early intervention. Ideally, an FA transplant team provides long-term follow-

up, even if the individual hasn't been transplanted, due to the complicated and particular needs of people with FA.

Long-term challenges of FA include endocrine, reproductive, nutritional, neurological, musculoskeletal, and psychosocial, as well as potential malignancies. Furthermore, treatments themselves - whether transfusions, androgens or transplant, can each have long-term effects.

During the first year post-transplant people are at very high risk of bacterial and viral infections. Children should not return to school for at least three months after transplant from a matched sibling donor, and for at least six months after an unrelated transplant. They will need to redo all childhood immunizations, starting one year post-transplant, or even later if they have chronic graft-versus-host disease (GvHD). Importantly, they should have *no live vaccines* for at least two years post-transplant. All patients and all family members should receive a flu vaccine every year.

Endocrine issues are very common in FA with over 80% of people having at least one abnormal endocrine test. Additional risks for endocrine disorders include chronic red cell transfusions, androgen therapy, midline brain abnormalities and transplant. Dr. MacMillan recommends yearly visits to an endocrinologist.

Malignancies are a particular concern as those with FA get older. While FA alone vastly increases the risk of malignancies, transplant increases the risk further - particularly radiation, chemotherapy and GvHD. All patients should receive the HPV vaccines (prior to transplant and

two years post-transplant). Although the association with HNSCC (head and neck squamous cell carcinoma) isn't as strong as previously thought, the vaccine may still prevent some of these cancers and some cervical and vulvar cancers.

People with FA can also reduce their risks of malignancies by refraining from drinking or smoking and reducing exposure to other carcinogens such as benzene and excessive sunlight. However, Dr. MacMillan also reminded the audience that it's important to live life! Realistic expectations, moderation, and education are key to a balanced life.

Most doctors aren't familiar with FA and aren't expecting malignancies at a young age, particularly HNSCC. Dr. MacMillan emphasized the importance of early and regular six month screenings, trusting your instincts if you are concerned about a potential malignancy, and seeing an FA specialist.

Quality of life is a very important aspect of living with FA. Many things can impact the quality of life and provide challenges at school and at work, such as neurocognitive deficits, anxiety, depression, social withdrawal, and insurance battles.

Dr. MacMillan stressed the importance of close monitoring and early intervention. Ideally, an FA transplant team provides long-term follow-up, even if the individual hasn't been transplanted, due to the complicated and particular needs of people with FA.

A common theme with each of these aspects of living long-term with FA is that it is important to continue regular visits to your FA specialists, and consult the *Clinical Care Guidelines* for specific recommendations. Dr. MacMillan left the audience with a key message: "Patients need to be followed for life!"

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Mind, Body, Spirit: Tips for Stress-Reduction



Richard Dickens, MS, LCSW-R,
CancerCare

For many, living with Fanconi anemia or caring for a child with FA means weaving a path through the throes of sickness, treatment, financial concerns, and the looming threat of cancer. This year at Camp Sunshine, one presenter acknowledged the reality of the worry inherent in life with FA, and offered a few stress-reduction strategies.

Richard Dickens described stress as a normal physiological response of the body to environmental threat. In fact, the body's natural "fight or flight" reaction to perceived danger is part of what allowed humans to escape predators and evolve over time.

Chronic stress puts people at risk for anxiety, depression, digestive issues, heart disease, sleep problems, weight gain, and memory/concentration impairment.

This reaction involves the release of stress hormones, which enable the body to respond quickly, for instance to run from a bear or mountain lion. However, chronic activation of the "stress pathway," particularly overexposure to cortisol, can be damaging physically and mentally over time. Chronic stress puts people at risk for anxiety, depression, digestive issues,

heart disease, sleep problems, weight gain, and memory/concentration impairment.

So, what to do about it? There are many ways to deal with stress and no one size fits all. Dickens offered ideas to help people find a place of peace, hope, strength, and balance. Please consider the following invitations:

- Try a meditation exercise (there are apps for these!): bring your attention to a positive mantra or follow the breath without judging the thoughts that enter your mind.
- Try creative visualization exercises. These can be especially helpful before medical appointments.
- Prayer can be deeply meaningful for those who belong to a religious or spiritual community.
- Get active! Exercise releases endorphins, natural mood boosters.
- Talk to a loved one.
- Eat well. Caffeinated drinks, sugar, and alcohol can contribute to stress.
- Drink water.
- Do one small act of kindness for another person.
- Write down three things for which you are grateful.
- Try movement-oriented forms of meditation, like walking meditation, qigong (chi gong), or yoga.
- Reduce time around technology.
- Get into nature!
- Experiment with drumming, a form of vibration therapy.
- Breathe! Try the three-breath exercise: Bring attention to your abdomen and take in a deep breath. Think of a balloon, filling the lower part of your belly first. Hold your breath for a count of seven and then release. Repeat two more times.

Study Underway to Detect Oral Cancer in FA

If you or someone in your family is diagnosed with oral cancer, please consider participating in a research study funded by FARF to determine if saliva can be an early detection tool for oral cancer. Contact FARF as soon as possible after diagnosis and before treatment at research@fanconi.org or 888-FANCONI. FARF will coordinate your participation with David Wong, DMD, DMSc, the study's principal investigator. For more information, visit Research Highlights on our website.

Ask the Experts Q & A

Family Meeting attendees had the opportunity to ask questions at an “Ask the Experts” session hosted by Dr. Blanche P. Alter and Dr. Richard Gelinas. Additional information was provided by Dr. Eunike Velleuer and Dr. Jennifer Adair.



*Richard Gelinas, PhD, Institute for Systems Biology, Fanconi Anemia Research Fund Scientific Advisory Board and Board of Directors;
Blanche P. Alter, MD, MPH, National Cancer Institute.*

Q: Is it better to use platelet transfusions or danazol to postpone transplant while trying preimplantation genetic diagnosis (PGD)?

A: It is always an individual decision. Because PGD takes time, it's wise to choose treatment options that are long-lasting. Platelets don't last very long, so you would have to transfuse roughly once a week. More importantly, platelet transfusions, like blood transfusions, can make a transplant more complicated. On the other hand, danazol is safe and doesn't jeopardize transplant when used appropriately, with monitored liver function and liver ultrasound. Androgens used to be considered a risk factor at transplant, but they have improved, the way we use them has changed, and transplantation itself has improved. Current data from multiple locations in the world shows that androgens are safe to use, even prior to transplant, if needed.

Q: Is immunotherapy (a type of cancer treatment designed to boost the body's natural defenses to fight cancer) being used in other solid tumors besides Head and Neck Squamous Cell Carcinoma (HNSCC)?

A: It was first applied to melanoma, sometimes very successfully. Next it was tried with lung cancer, both small-cell and non-small-cell, and now it has been tried with kidney, liver, and some other cancers.

There are three ways of redirecting your T-cells:

1. Small-molecule regulated immunotherapy
2. Gene therapy to target the tumor - this has been done with blood cancers and now solid tumors, too
3. Adoptive cell transfer therapy (pioneered by Dr. Steven Rosenberg, National Cancer Institute) - tumor-infiltrating (TIF) white blood cells are extracted from the tumor, expanded to large numbers outside the body and reinfused in the patient while he or she is given immune boosters. This technique is being tried in a wider variety of tumors than the other strategies.

Scientists are currently exploring whether the immune system can be used to eliminate the bone marrow prior to transplant instead of using chemotherapy and radiation.

Q: We have heard about collaborations between FA researchers, and the Fund convenes the Scientific Symposium as well as some special-topic meetings. When the Fund awards grants, are researchers required to share with others?

A: Yes! We take pride in the wisdom of that policy. Additionally, 20 years ago, the Fund established a repository for cells and antibodies. This has been invaluable to researchers and is a wonderful way to boost collaboration.

Help Advance FA Research!

Researchers are working hard to find effective treatments and a cure for Fanconi anemia, but they can't do it alone. FA researchers need you. Please consider donating tumor tissue for FA research. Post-mortem tissue donations are also invaluable in helping us understand and treat the cancers that affect so many individuals with FA.

For more information, contact FARF at research@fanconi.org or 888-FANCONI.



Giving Back

By Donna Boggs



My husband and I continue to return to Camp Sunshine year after year to try to give back for all the wonderful, fun-filled memories that camp gave

to our family during the roughest times of our lives. I'm not sure that we will ever be able to repay the debt that we owe. We keep racking our brains for projects that will improve the experience of camp! How do you improve something so wonderful to begin with? How do you make trips to camp even more special than they already are?

Our sons, Nicholas and Spencer, loved to go to Camp Sunshine! Nicholas would hopscotch from one holiday to the next until it was time to go back. He loved being the center of attention! It is truly a magical place and we wanted to build something in his memory. How on earth could we ever capture what camp meant to him? I don't think we ever will.



Our first year back to camp after Nicholas passed away from leukemia in October, 2010, was the hardest. We arrived a few days early and it was wonderful to be alone to grieve and to laugh at all the memories. We really needed that time to heal and to reflect on what his love for life meant to us and to camp. Our first project was to create a magical spot to do just that. We built "Nic's Nook" and "Spencer's Space", arbor benches overlooking the lake, a place to seek peace, comfort, and solace, while enjoying the beauty of Sebago Lake. The benches have become a favorite place among the families as well as the volunteers; a place to meditate, de-stress, read, pray, rejuvenate, grieve, and just enjoy the beauty of camp.

While building the benches, we were contacted by Holly Hoxeng. She wanted to make a donation and wanted us to choose the project. I walked by the playground and remembered all the times that Nicholas wanted to swing and play. He could only stay out for short periods of time because there wasn't any shaded place to play or swing. Keeping in mind the sun's role in cancer issues for Fanconi anemia patients, we installed shades in a couple of areas for the children to play under. Now, there are also benches under the shades for the parents to watch their children play, making it much more enjoyable for families.

After this project, the folks at Camp Sunshine realized Jeff's ability to build, and since then, they have put him to work every year! The following year, he built the Grape Arbor for Anna Gould's family garden. He used cedar, which not only enhanced the look, but gave a wonderful aroma as well.

He has also repaired the frames for the horseshoe pit and Bocce court and built park benches to place throughout the camp. Camp Sunshine liked the benches so much that he was asked to build more, as well as Adirondack chairs to place in the Fanconi Garden. Jeff's bench design was even replicated





and built from cedar for the Lawrence Gould garden. What a beautiful tribute to Mr. Gould.

As you can tell, the projects keep getting bigger and bigger! This year, he built a covering over the outdoor kitchen. With the help of Camp staff, he had it built in time to be christened at the BBQ on Monday during the Family Meeting.

The final project for this year was a cedar bench and table in memory of Dave Frohnmayer, to be placed in the Fanconi Garden. This is such a small tribute to pay for the monumental effect Dave has had in the FA world. Thank you, Dave, for being you!

And all of this began with one small gift from God who blessed our home for 12 years. The greatest gift to us was his unconditional love. He LOVED everyone. As we strive to portray that love throughout camp, we hope all who visit Camp Sunshine use and enjoy our projects.

With love,
The Boggs Family



Transitioning to Adulthood



Hello! My name is Karly Ross and I am a 20-year-old with Fanconi anemia. I was diagnosed in 2002 at age seven and had a bone marrow transplant at the City of Hope (MUD BMT) in March 2003. I am currently a sophomore at the University of South Carolina at Lancaster

and am studying nursing. I hope to become a pediatric oncology nurse!

As an adult with FA, I am transitioning into taking care of my health on my own. My first encounter with the transition into adulthood was attending a "Survivorship Clinic" in 2013. Instead of being seen by the pediatric oncologist, I met with a team of specialists in adult oncology, psychology and

nutrition. In 2014, I traveled to Baltimore, Md. to attend my first meeting for adults with FA. I met many new friends, all of whom were older than me. While at the adult meeting, I met a woman who was 60 years old, who gave me and a lot of the other FA adults hope for our future.

Transitioning into an adult patient has not been the easiest thing in the world because it comes with its own challenges, such as the new responsibilities of making your own appointments and remembering all of your medications, not to mention everything else that comes with being an adult. My mom always took care of the medical stuff for me, but as an adult, I need to take ownership of my own health. I believe that it is somewhat easier to cope with FA as an adult because you are at a point when you can—for the most part—understand what is happening to you and you can empathize with other patients. I know that I am not alone in my struggle with FA. However, along with adulthood and this understanding, it becomes that much harder to lose a friend to this disease. We are just one giant family. I am not letting FA stop me from living a long and fulfilling life!

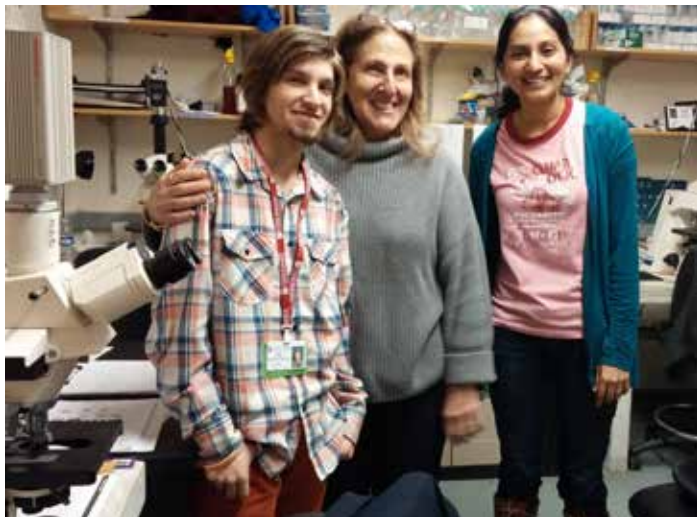
From High School to Harvard

By Matt Pearl



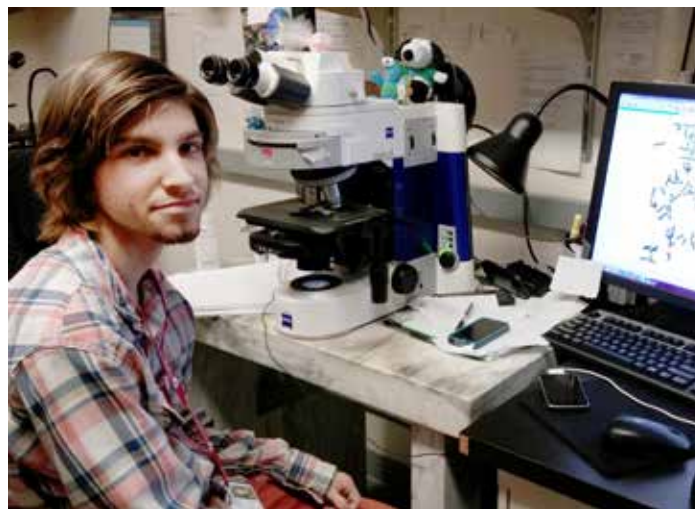
I was diagnosed with Fanconi anemia on Jan. 14, 2001. I will never forget that day because it was my fourth birthday. Yes, FA can be extremely complicated and confusing, but I would not change my life. I am now 18 and have just started college at Westminster College in Fulton, Mo., and I have a sister, Alexandra, 20, who also has FA. We have both been successfully transplanted from unrelated donors and live amazing lives. I am studying psychology, nonprofit management and motivational speaking. I learned at an early age that life is what you make of it and has a lot to do with being in the right place at the right time. I feel that for people with FA, living fully is about focusing on what you *do* have, not on what you *don't*.

In my case, it was sitting in a medical meeting at Camp Sunshine, listening to Dr. Alan D'Andrea's presentation,



when I had this reflection. At the end of his talk, he explained his intent to have an FA patient work in his lab at the Dana-Farber Cancer Institute in Boston, Mass. I marched up to him and said, "I am not a brainiac, but I would like to make a difference helping with FA research." That is how I became the first FA patient in the world to go to Harvard before college and work with Dr. D'Andrea.

I graduated high school early in December and left for Boston to make history. Well, at least weather history, as it was a record year for cold temperatures and snow. Did I mention that I walked about a mile each way to the lab every day? Was it worth it? YES! I quickly learned that in Boston, no one waits for the walk sign at a crosswalk in the freezing cold.



It was an amazing experience. I learned about molecular pathways, somatic mutations, cellular sensitivity, DNA repair and, admittedly, I sat through a few lectures that went right over my head. I worked with many researchers who had never met, let alone interacted with, a *live* person with FA on a daily basis. Many said that working together changed their lives and motivated them even more to find answers. Dr. D'Andrea and his lab, especially Lisa Moreau, Director of Cytogenetics, are so inspirational and beyond dedicated to the FA community.

It turns out I inspired them as well. Dr. D'Andrea said, "I met Matt Pearl at Camp Sunshine in 2014 and was so impressed by his leadership skills. I was delighted to have him join our laboratory. He worked in our chromosome breakage lab and had such a motivating presence."

International Fanconi Anemia Day

By Peg Padden and Mary Ann Fiaschetti

FA Families started International Fanconi Anemia Day (May 1st) in 2010, as a day to raise money for much needed FA research. ALL money raised, no matter how small the amount, was important, because every little bit added up to other little bits, to make the difference we were looking for. School Penny Drives, Yard Sales, Chess Tournaments, Turtle Races, Family Fun Days, Bunco, Bowling, Auctions and more, all took place during the month of May, raising a total of \$135,000. The next two years brought similar results, resulting in a three-year total of approximately \$350,000!

A few Families raised money in the next couple of years, and many posted photos on Facebook to bring about FA awareness. Awareness is a much-added bonus, but our primary goal is of course to raise money for research. That is why Mary Ann Fiaschetti got the ball rolling again this year, setting up Crowdrise, a simple and easy way to raise money online.

We are happy to say we had 27 Families from 14 states and five countries raise a total of \$150,000 this year, which is truly fantastic! And there's one thing I know for sure: We will not let any more years go by without keeping this up. We can't. Together we will find a cure.

Fundraising made Easy

When David Fiaschetti signed up for the October 2014 Pell Bridge Run in Newport, R.I., he was given an opportunity to fundraise for a non-profit organization of his choice. Crowdrise was the mechanism the race organizers offered to fundraising participants. With ease, David set up an event page designating the Fanconi Anemia Research Fund as the recipient of donations in honor of his son, Peter. It only took a few minutes to establish the link and share via social media and email with family and friends. Noting how conveniently Crowdrise automated the usual hassle of fundraising tasks, he and his wife, Mary Ann, immediately wanted to share this fundraising tool with other FA families.

In March 2015, just two months before the 6th



International Fanconi Anemia Day, Mary Ann offered to assist FA families interested in fundraising by setting up the FA Day Event on Crowdrise, inviting everyone to post an individual FA Day fundraiser, and then donating \$25.00 to each fundraiser posted on Crowdrise. Seventeen Families utilized Crowdrise for their FA Day fundraiser.

Crowdrise allows the user to set up a personalized page with text, photos and videos of the fundraising activity. By selecting the Fund as the nonprofit recipient, the donations are sent directly to the organization. Crowdrise immediately notifies the organizer of any donation, and with a click of a button, sets up a reply thank you email to the donor. The site offers great technical and personal support. If you are interested in using Crowdrise for a future fundraising event, feel free to email Mary Ann at faday4cure@gmail.com.



Donate While You Shop on Amazon

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

Rising to the Challenge

By Mary Ann Lana



It was mid-January 2015 when a post from fellow FA mom, Peg Padden, appeared on the Facebook Family Support page. Peg was putting an urgent plea out to parents to increase their fundraising efforts for Fanconi Anemia Day on May 1. I was reeling from the loss of Piper Bentley just 24 hours before, and had just completed a pretty successful birthday fundraising event for our son Eli, but I knew in my heart I had to do more. Peg's words, "If we don't, who will?" really hit me. I commented on her post that I had long wanted to do a 5K in my hometown, but wasn't sure where to start or if I could even pull it off in the amount of time I had with no 5K experience. Peg assured me I could, and lucky for me, she'd organized a successful 5K herself and had a PDF guide for me to use, conveniently posted on FARF's website. So with that, this full time teaching FA mom with no 5K experience, dove in headfirst. Our first annual "5K for FA" was underway.

The first thing I did was fire off an email to a dozen of my closest friends to ask them for their commitment. We would have monthly meetings; we would each be in charge of some aspect of the event; we would pool our connections; we would figure this out together. Everyone was in. All I knew for sure at this point was that 1) Any amount of money we raised would be considered a success; 2) We would not spend any of our own money unless absolutely necessary; 3) We would appeal to the hearts of our community members by essentially using Eli as our "brand." That idea may sound cold and removed, but read on. It's actually quite the opposite.

On this journey I learned a few things about fundraising. I learned that if you start with why, you develop a project

that has heart and soul. "Why are we having this 5K?" To help this kid right here, and all those like him. We educated people about FA by tying it all back to Eli and his FA friends. We put his actual handprint on letterhead, signs, address labels, and shirts. It became an event about a boy, not about raising money for a faceless charity. I sent wallet pictures with correspondences and got a volunteer marketing team to use his pictures and hand logo to advertise the event. Not only that, but I had support from plenty of other FA families, too. I received about 50 pictures of FA kids that I printed and hung across the stage in a highly visible spot. I invited FA families to attend and was THRILLED that three of them could come, to mix and mingle and to just be there, visible. In fact, Andre Hessels, father of FA kids Joy and Dylan, won the race with the best time! Sorry about the course marshal leading you in the wrong direction, Andre. We'll make sure that is fixed for next year!

The main idea of the 5K was not so much the event itself, but about coming together as a community to do something important. In order to make that happen, we had to make sure that the event was highly organized and that it would run seamlessly. I met with many, many run experts in our area and polled a lot of people who participate often in these kinds of events. I compiled lists of things we had to do and obtained all of the legal permits. Once the ball got rolling it took on a life of its own, and people started contacting me asking how they could help!

We had a sponsorship program. We sent a letter to about 200 local businesses asking for donations in exchange for advertising on our shirts, website, program, and signs.





This alone was a huge success. We further requested that participants raise \$100 or more each. By and large, many of those 132 people did that and more. And did I mention the t-shirt sales and 50/50 raffle? We even had FA mom Aliza Canonica send handmade keychains from Switzerland to sell. Simply put, I gave people a lot of fun, creative ways to part with their money, and to do so with a smile on their faces.

On race day, I spoke to the crowd about Piper Bentley. The fact that her parents were there standing behind me at the FARF booth was an overwhelmingly emotional moment that was beyond powerful. “People give to people, not things,” was my driving mantra. We made it about the people who are affected: normal, everyday, average families. The Hessels, Bentleys and Collings families were present, and helped

people realize that as much as this event was about Eli and our family, there are a lot of other families just like ours that need help, too.

At the close of the event, we had brought in about \$12,500 for the Fund. More than that, we made about 300 people feel like they were a part of something big and meaningful. I had droves of participants praising the organization of the event and the course itself, but more than that, I heard again and again that people loved feeling like they were a part of something. That, to me, was the blessing. We raised a ton of money at our first race and we helped people feel like what they did that day mattered. And it did.

Lastly, I really want to thank this very special organization for partnering with us through the duration of our “5K for FA.” From the early panicked planning stages, to the materials and signage they provided, to the financial help and record keeping assistance. We could not have had such a successful event without the help from the Fund. Thank you, FARF!

For the fundraising events calendar and helpful fundraising materials and tools, visit www.fanconi.org

FARF Can Help You Fundraise

More than 90% of the Fanconi Anemia Research Fund’s annual budget comes from family fundraising. We’re here to help make your events a success. We can:

- Provide sample fundraising letters and help you edit your letter
- Use your photos to personalize your letter, event invitation or brochure
- Use your mailing list to send your letter or invitation from our office
- Provide ideas, information, and display materials for events
- Provide a PowerPoint or video presentation to use at your event
- List your event on our website

- Send a thank-you letter and tax receipt to your donors
- We ask that all fundraising events be covered by liability insurance. Insurance for a one-time event is often available through a family’s homeowner’s insurance policy as a relatively inexpensive insurance rider. Please contact the Fund if you need assistance obtaining or paying for this required insurance.

Please ask your donors to make checks payable to the Fanconi Anemia Research Fund. When a donation is received, we’ll generate a letter of thanks with a tax receipt, and we’ll notify you that a donation has been made in your behalf.

We appreciate all your efforts to raise funds for FA research and family support. You are making a difference!

Honoring a Legacy

By The DeHaan Family



How do those of us who have lost loved ones to Fanconi anemia keep hope alive and keep waging our battle against this disease? What involvement will we continue to have when our primary reason for fighting is gone? These questions were very daunting for us as we approached the one year mark following our son, Cooper's, passing.

After spending almost six years fighting alongside Cooper as he spent countless months in the hospital, overcame leukemia, underwent a successful bone marrow transplant, and finally yielded in his fight after a leukemia relapse six months post-transplant, we struggled with the question "What now?" While we knew that Cooper (*FANCD1/BRCA2*) would likely face massive challenges at some point in his life, we were not prepared for the abrupt end. He spent his short life bravely battling FA with a warrior's spirit and with unfaltering will. Having successfully defeated everything

How would we fundraise *without* Cooper, but still do it *for* Cooper?

thrown at him previously, we were certain that we had extended our time together as a family again. On Aug. 17, 2014, Cooper passed peacefully into the everlasting comfort of God's care.

While we struggle to understand the meaning of everything Cooper had to endure, we know that we have to move forward, in the same way Cooper showed us his entire

life—one day at a time, one battle at a time. How could we possibly STOP fighting against a disease that took our son and that other "Coopers" continue to endure? While Cooper will no longer be with us on Earth, we feel a tremendous duty to honor his memory through the extension of his battle against FA. Remaining connected and involved with our FAmily helps keep our son at the surface of our lives and memories of our time with him strong. He positively affected so many people in such a short amount of time with us. As parents, we are immensely proud of his impact on others—his joy, his courage, and his gift of laughter through even the toughest times. It is not a choice, but an obligation that we have as his parents to continue his impact and to strengthen his legacy, even in his physical absence.

Cooper had been gone from us for nearly a year when we started to wonder how we would continue to raise awareness and funds for the Fanconi Anemia Research Fund without having him by our side. How would we fundraise *without* Cooper, but still do it *for* Cooper? While we brainstormed new fundraising ideas, we quickly decided that we needed to create a memorial fundraiser, in Cooper's honor, to benefit the Fund. Concluding on the date that marked one year of his passing, Camp Cooper's Memorial Fundraiser would raise \$2 for each of his 2,038 days on Earth. The goal: \$4,076. We decided to make his memorial fundraiser simple and easy. Within a day, a GoFundMe page was created and shared with "virtual" friends – a tightknit online discussion community that had supported us and had closely followed Cooper's final months on Earth. This was an effective way to broaden our fundraising network without asking the same (non-virtual) family, friends and colleagues to give. It also gave us something worthwhile to do while we planned our next event. The result was outstanding. Members spread the word, donated personal goods for an online auction and generously gave financial resources. On the official one year mark, nearly \$5,000 had been raised for the Fund in Cooper's memory.

On a personal level, the benefits of our refreshed involvement within the FA community are many. Not only are we able to continue Cooper's fight for others battling FA and for those searching for a cure, it also assures our unending connection with Cooper and his legacy.

My Best Friend Jr.'s Carnival for a Cure

By *Chloe Rogers*

My name is Chloe Rogers and I was diagnosed with Fanconi anemia when I was 13 months old. Our family's closest friends began asking how they could help. A wonderful group of my mom's friends banded together to form a committee. They called themselves "My Best Friend." They threw three colorful fundraisers called the "Cinco de Mayo Fiestas for FA," and raised over \$550,000 for the Fanconi Anemia Research Fund.

I'm grateful to FARF for the life-saving research they've funded that enabled my bone marrow transplant to be a success. I watched as the Fiestas for FA were planned, and around Christmastime I decided I'd like to help fund research, too. I sent a letter to my friends and asked if they'd like to start a group called "My Best Friend, Jr." and do a fundraiser for FARE. Our first committee meeting was in January 2015 and I was so grateful that 35 kids (and some 'kids-at-heart') came, full of enthusiasm and energy. After brainstorming for ideas, the "Carnival for a Cure" was born!

Together with my friends, we asked to borrow old-fashioned carnival games from the Lawrence Beach Club. Looking to appeal to all ages, we picked games like "crush the can," balloon darts, bean bag toss and a goldfish game. We ordered a multitude of great prizes for winners, and had fabulous raffle prizes as well, such as a surf board, jewelry and gift certificates.

For the main event, a group of terrific turtles entertained the carnival goers by participating in a series of races. The



Turtle Derby, as it was called, was inspired by the one I watched during my transplant at the University of Minnesota Children's Hospital. After a lot of research on turtles, we found Jungle Bob's Reptile World, which generously loaned us turtles and an expert for our Carnival. By donating \$100, families could name the turtles. The hysterical names and turtle antics entertained a cheering crowd. The Turtle Derby was arguably the best event at the carnival and definitely the most unique.

On April 25, 2015, people in our community attended the "Carnival for a Cure" at a local event hall. Turtles raced, prizes and raffles were won, faces were painted, and a net total of \$24,000 was raised for FARE. I'm over the moon that my friends were so supportive. They made this event something I'll never forget.

In Loving Memory

"For some moments in life there are no words."

Tali Rom9/9/86 – 4/1/15

Justice Spencer5/4/06 – 6/28/15

Trayonia Brown.....1/21/83 – 4/8/15

Ashley Ross.....4/16/87 – 8/1/15

Anneme' Rossouw4/4/95 – 4/26/15

Niko James Peros5/4/12 – 8/9/15

Israel Becerra.....1/8/09 – 6/28/15

Aria Alaniz Gatzlaff1/12/11 – 9/20/15

Family Fundraising Efforts January through August

From January 1 through September 1, 2015, Fanconi anemia families raised an incredible \$1,199,866 for the Fanconi Anemia Research Fund. Almost 89 cents of every dollar donated goes directly to research and family support to make a difference in the lives of individuals and families affected by FA. Thank you for your outstanding fundraising efforts so far this year!

\$213,000 – \$331,000

Dave, Lynn, and Amy Frohnmayr
Kendall & Taylor Atkinson
Foundation with the Nash and
Atkinson Families

\$54,000 – \$72,200

Steve and Jennifer Klimkiewicz
Kevin and Lorraine McQueen

\$21,000 – \$50,000

John and Kim Connelly
Matthew and Evelyn Keyes
Todd and Kristin Levine
Kevin, Katie, and Chloe Rogers
Glen Shearer and Peg Padden
Gerard and Cynthia Vandermeys

\$10,000 – \$20,000

Jimmy and Jenny Armentrout
Owen and Cindy Bagaason
Kerrie Brannock
Mark De Groot and Hanneke
Takkenberg
Claudia Fernandes
Tim and Mary Ann Lana
Orion and Lisa Marx

\$5,000 – \$9,999

Kaps for Kendall
Chris and Susan Collins
Susan and Skip Gannon-Longstaff
David Guidara and Ceresa Family
Brian Horrigan and Amy Levine
Charles and Katy Hull
Andries and Helga Kruger

\$1,000 – \$4,999

Michael and Jennifer Aggabao
Rachel Altmann and Tyler Morrison
Israel and Mary Jo Becerra
Randy and Nancy Bloxom
Jeffrey and Donna Boggs
Chris and Jennifer Branov
Sean and Allison Breininger
Ryan and Becky Brinkmann
Robert and Barbara Capone
David and Kim Chew
Tom and Mary Eileen Cleary
Daniel and Melinda Coleman
Ana Concha
Darrel and Kalani DeHaan

Ezat and Laila Faizyar
Justin and Britteny Ferrin
David and Mary Ann Fiaschetti
Andrew and Jennifer Gough
Alan and Rachel Grossman
Owen Hall and Margaret Kasting
Andre Hessels and Rutger Boerema
Daniel Kold
Mark and Angela Lamm
Gregory and Lynnette Lowrimore
Dan and Nikki McCarthy
Sheila Meehan
Adam and Olivia Mondle
Bob and Alice Nicholson
Susan Ortiz

Mark and Diane Pearl
Michael and Joanna Peros
Peter and Janice Pless
John and Dianne Ploetz
Joel and Jennifer Ramirez
Chad Ramser
Pedro and Marina Ravelo
Mark Ritchie and Lisa Mingo
Les and Nancy Ross
Bob and Andrea Sacks
Ron and Elesa Schaefer
Bryan and Karen Siebenthal
William and Mary Underriner
Mike and Beth Vangel
Louis and Theresa Viola
Nigel and Ann Walker
Robert and Julie Williams

Up to \$999

Al and Janeth Acosta
Lou Amico
Brian Anderson and Sultana
Graham-Anderson
Ron and Juanita Arroyo
Ken and Jeanne Atkinson
Cherie Bank
Gerald and Julie Barbier
Mark and Linda Baumiller
James and Tracy Biby
Darryl Blecher and Diana Fitch
Richard and Tena Boson
Dale and Chris Bossy
Donald and Danielle Burkin
Patti Carter

Bradley Curry and Lea Ann Stiller
Paige D'Angelo
Donna DellaRatta
Scottie and Jessica Dill
Antonino and Marie DiMercurio
David and Kari Doctor
Brian and Jennifer Dorman
Jonathan and Sharon Drew
Ed and Janice Duffy
David and Kelly Dunnock
Mir Saleem and Umber Ellahi
Sharon Ellis
Billy Jo and Debbie Estep
Emily Estes
Edwin Ferreira
Liz Funk
Gary and Melody Ganz
Kevin Gatzlaff and Rachel Alaniz
Brian and Lisa Gillott
Anthony Glavac and Sabrina
Bowmer
Pat and Maria Gleason
Josh and Maria Godwin
James Grady
Michael Greenberg
Doreen Gummo
Abdul Hameed
Jackie Hardy
Bob and Victoria Hathcock
Greg and Diane Hayes
Nancy Heiligman
Jeff Hoffman
Bonnie Hutchins
Shane and Colleen Irvin
Jeff and Beth Janock
Kayla Lackey
Christopher and Dana Lamb
Martin Lamo
Eddie and Maly Lee
Peg LeRoux
William and Amy Lewis
Tanner and Jessica Lindsay
Eric and Beth Losekamp
Donnie and Jerri Lott
Bill and Jackie Lucarell
Stuart Cohen and Deane Marchbein
Tue Marker
Kevin and Barbara McKee

Catherine McKeon
Gianna and Lauren Megna
Jim and Holly Mirenda
Ian and Tricia Mitchell
Tony and Lina Nahas
Louis and Virginia Napoles
Jack and Lisa Nash
Jack and Tammy Neal
Robert and Mary Nori
Ron and Fredi Norris
Fred and Nancy Nunes
Michael and Katharine Ormond
Joshua and Crystal Pepper
Lori Petersen
Lynn and Shirley Quilici
George and Kathryn Reardon
Leonard and Jan Riley
John and Traci Robbins
Emily and Neil Robison
Stanley and Lisa Routh
Rick and Lynn Sablosky
Mike Sanders
Richard and Dolores Satterlee
Chris Scaff
William Schaecher
Bill and Connie Schenone
Thomas and Brenda Seiford
Helen Severson
Jack and Debbie Siegel
Cearra Stanec
Adam and Jennifer Stewart
Charles and Jennifer Sumrall
Mary Tanner
Peggy Templeton
Bruce and Loreen Timperley
Mark and Susan Trager
Tom and Kathy Uno
Abid and Reshma Shahid Usmani
Roma Vangel
Joe and Wendy Vitiritto
Carson Vitrano
Elizabeth and Graham Walker
Marc Weiner
Welfare Family
David and Erica Williams
Michael and Kim Williams
Brenda Witherspoon
Sean and Kristin Young



Making Strides for FA

In honor of Fanconi Anemia Awareness Day and Month, Peggy Padden strapped on her hiking boots, loaded her backpack, and headed out to the Pacific Coast Trail. She and her friend spent Memorial Day weekend hiking in memory of Peggy's son, Jake, whose zest for life and passion for the outdoors spurred them forward on their trek.

"I knew Jake would think it was 'cool!' and flash his big Jake smile," Peggy shared. "I miss him every day and only wish he could have joined us."

As she made strides on the trail, Peggy raised a total of \$2,667 to help make strides in Fanconi anemia research for her son, Spencer, and others with FA. Way to go, Peggy!



Hiking for Hope

The Fiaschetti family took to the outdoors on Memorial Day to raise funds for the sixth annual Fanconi Anemia Day. Hiking 10 miles through the serene pastures of the Sakonnet Greenway Trail on Aquidneck Island, R.I., they remembered the FAmily members who have touched their hearts, and fortified their hope for advancements in medical research.

"We fundraise because our son, Peter, deserves the same opportunity as any other child to reach his goals of a career, adventure, and love. Only by raising money for research, leading to better treatments and a possible cure, will he be guaranteed a level playing field," Mary Ann Fiaschetti explained.

The Fiaschetti family not only held their own fundraiser, they also encouraged other FAmilies to raise funds and awareness in honor of FA Day. Mary Ann inspired others through her call to action and continued support and guidance to FAmilies throughout the month. In addition, the Fiaschetti's graciously donated \$25 to every fundraiser! Thank you, Mary Ann and family, for your motivation, compassionate support and dedication.



Mario Kart Marathon

Did you know you could raise money for Fanconi anemia by playing games online? That's exactly what Daniel Kold did this past May in honor of FA month. Inspired by others with FA, Daniel played Mario Kart 8 for 24 hours straight!

Using the world's largest gaming site, Twitch.tv, he was able to play live while other gamers from all over the globe watched and chatted with him. During this time, he explained what FA is and what the Fanconi Anemia Research Fund does to help those affected. He answered questions and encouraged donations, raising a total of \$1,065! Good game, Daniel.

Weekend Getaway

Everyone could use a weekend away once in a while. To raise awareness about Fanconi anemia and to support research, the Breininger family generously offered a weekend getaway to their cottage on Lake Superior. "We want to spread the word about FA, help support research, and enable the organization to continue providing excellent support to families," Allison Breininger said.

For every \$50 donated to the Fund from May 1-20, donors were entered into a raffle to win the getaway. Twenty-seven people participated and the final amount of money raised was \$1,575! In the end, one of Allison's co-workers won the weekend to Lake Superior. Congratulations! And well done, Breininger family, for a fun and unique fundraiser!



Meet Pamela Norr



Pamela Norr, Fanconi Anemia Research Fund Executive Director

The Fanconi Anemia Research Fund is very pleased to introduce Pamela Norr, who has been hired as executive director effective October 5. She succeeds Laura Hays, who resigned in spring 2015.

Pam comes to the Fund with 20 years of senior healthcare and nonprofit leadership experience, including most recently as Executive Officer of Oregon Rural Healthcare Quality Network (ORHQN), supporting Critical Access Hospitals throughout the state by quality benchmarking, case review and culture change for patient safety. Prior to ORHQN, she was the Executive Officer for Central Oregon Council on Aging for five years, helping seniors stay independent and secure. Her

other experience includes a variety of senior management positions in hospital administration in nonprofit hospitals in California.

Pam received a BA in English from the University of Oregon and is a very active volunteer in community causes. She has served as a member on several boards and as a volunteer for many organizations, including the American Cancer Society, Alzheimer's Association of Oregon, and the National Cooperative of Healthcare Networks.

She and her husband, Tom, have two daughters, one 20 and one 17, and one very persnickety Westie dog. She enjoys travel, both domestic and international, and writing.

Excited to join the FARF team, Pam said "I am looking forward to getting to know the families, the scientists, the staff and the board, to work together as a team to support those affected by FA, and to increase fundraising to better support research."

Your FA Research Dollars at Work

From April to September 2015, the Fanconi Anemia Research Fund awarded \$518,094 in research grants to the following projects:

Investigator: Ian Mackenzie, BDS, FDSRCS, PhD, Queen Mary University of London, London, United Kingdom
 Title: *The effects of loss of Fanconi gene function on the behavior and therapeutic responses of head and neck cancers*
 Amount: \$90,886

Online Fundraising Tools Available

Qgiv and Hobnob are online fundraising tools available through the Fanconi Anemia Research Fund. Through Qgiv, we can accept online donations directly on our website. Hobnob offers people a customizable fundraising page for events, enabling online registrations and donations in advance and at the event. Contact FARF for details on how Qgiv and Hobnob can enhance your fundraising!

Investigators: Ashley Kamimae-Lanning, PhD; Ketan Patel, MD, PhD, Medical Research Council Laboratory of Molecular Biology, Cambridge, United Kingdom
 Title: *The Role of Aldehydes in Fanconi Anemia Oral Squamous Cell Carcinoma*
 Amount: \$171,000

Investigator: Nicholas T. Woods, PhD, Eppley Institute for Research in Cancer, University of Nebraska Medical Center, Omaha, Neb.
 Title: *Deciphering the endocrine-specific role of FA proteins in pancreas islets*
 Amount: \$175,000

Investigator: Markus Grompe, MD, Oregon Health & Science University, Portland, Ore.
 Title: *Bridge Funding for Metformin Studies*
 Amount: \$81,208

The Fund is committed to supporting research to further our mission of finding new treatments and a cure for Fanconi anemia. Over our 26-year history, we have funded 206 research grants and one service grant to 109 investigators worldwide. The total amount of research dollars awarded is over \$17 million!



The Fund Achieves a Four Star Rating with Charity Navigator

The Fanconi Anemia Research fund has been ranked “exceptional” (four stars) by Charity Navigator, the largest independent reviewer of charitable organizations in the US. The ranking comes after a review of our 2014 financials by the charity watchdog organization.

Charity Navigator evaluates the financial health of more than 5,000 charities—reviewing day-to-day operations, sustainability and accountability. The Fund keeps administrative costs low, allowing more money to go to research and family support.

SAVE THE DATE
FEBRUARY 26-29, 2016
 Register by 1/20/16
 Travel scholarship
 deadline 12/15/15
ORLANDO, FLORIDA
 ROSEN PLAZA HOTEL
MEETING FOR ADULTS WITH FA
 For adults 21+ Adults 18-20 accepted on case-by-case basis
 Fanconi Anemia
 RESEARCH FUND, INC.

PayPal Giving Fund

Do you want a simple way to increase giving to the Fanconi Anemia Research Fund? Welcome to the PayPal Giving Fund.

EBay sellers are encouraged to give a percentage of their proceeds to a nonprofit certified by PayPal Giving Fund each time they list an item for sale. Participating sellers are rewarded for their generosity with special eBay Giving Works features. The nonprofit receives recognition in the

listing and benefits from the seller’s success. PayPal Giving Fund and eBay collects and distributes the donation, and handles the tax receipt.

EBay members can also choose to make an online gift with PayPal. The Donate Now tab lets anyone with a PayPal account donate. For more information, see www.paypalgivingfund.org/index.html.

RETURN SERVICE REQUESTED

Mission: To find effective treatments and a cure for Fanconi anemia and to provide education and support services to affected families worldwide.

Use of Logo

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

Editors' Note and Disclaimer

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

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HOW YOU CAN HELP

- Donations Online:** Donate via the heart button on the Fund's website (www.fanconi.org) or through www.networkforgood.org or www.paypal.com
- Donations by Phone:** Call us at 541-687-4658 or toll free at 888-FANCONI (888-326-2664) (USA only)
- Donations by Mail:** 1801 Willamette St., Suite 200, Eugene, OR 97401

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