



April 2012

FA Family Newsletter

issue 51

A semi-annual publication of the Fanconi Anemia Research Fund, Inc.

23rd Annual Scientific Symposium Draws Record Attendance

The 23rd Annual Fanconi Anemia Research Fund Scientific Symposium, held last October in Barcelona, Spain, attracted 301 delegates from 24 countries—a record attendance due, in part, to an active FA research community in Spain and the relative ease of traveling to this Mediterranean seaport from other countries in Europe, Northern Africa and Asia.

In nine sessions over two and a half days, 48 researchers presented oral abstracts and an additional 84 researchers gave poster presentations on numerous aspects of Fanconi anemia. Jakub Tolar, MD, PhD, University of Minnesota, Minneapolis, chaired a special session on gene therapy, embryonic stem cells and induced pluripotent stem cells (iPSCs). This session included both a keynote address by Suneet Agarwal, MD,



Christophe Bichet speaks at the gala symposium dinner.

PhD, Children’s Hospital Boston, titled “Modeling Bone Marrow Failure Syndromes Using iPSCs,” and a panel of gene therapy experts from Spain and Germany. Maura Gillison, MD, PhD, The Ohio State University, Columbus, and a leading researcher on the relationship between the human papillomavirus and head and neck cancer, gave a special address in a session devoted to FA and cancer. Alan D’Andrea, MD, Dana-Farber Cancer Institute, Boston, spoke about “Fanconi Anemia and Novel Drug Targets,” and Francis Cucinotta, PhD, US National Aeronautics and Space Radiation Program, Houston,

gave a keynote address titled “DNA Damage Signals and Space Radiation Risk.”

The gala dinner, attended by all delegates, was an evening of celebration and inspiration. Hans Joenje, PhD, Free University Medical Center, Amsterdam, Netherlands, received a Lifetime Achievement Award, given just five times in the Fund’s 23-year history. Dr. Joenje is a pioneer in FA research whose discoveries provide the cornerstone on which virtually all FA research is based.

Two long-time FA researchers received the Fund’s Distinguished Service Award:

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- Juan Bueren, PhD, CIEMAT, Madrid, leads a cooperative FA research environment in Spain and is one of the global leaders in the FA gene therapy movement.
- Christopher Mathew, PhD, King's College, London, UK, has made significant contributions to FA research in addition to providing years of thoughtful insight as a member of the Fund's Scientific Advisory Board.

Three researchers received awards for their poster presentations:

- Best Basic Science Abstract: Koichi Sato, graduate student, Waseda University, Tokyo, Japan.
- Best Clinical Abstract: Kasiani Myers, MD, Cincinnati



Researchers discuss findings at the poster presentations.

Children's Hospital Medical Center, Cincinnati, Ohio.

- Best Translational Abstract: Jean Soulier, MD, PhD, Hôpital Saint-Louis, Paris, France.

Christophe Bichet, 26, gave an inspiring account of living with FA after dinner guests viewed a documentary film about him. His parents, Marie-Pierre and Charles Bichet, were in attendance and also appeared in the film speaking about Christophe's life-long interest in climbing rocks and jumping off—and of his living life to its fullest.

Evaluations received after the symposium confirmed the meeting's success. One attendee described the meeting as "excellent and inspiring" and said that the meeting "will certainly stimulate scientists to direct their research towards clinical applications to the benefit of FA patients." That's precisely the goal of every science meeting the Fund hosts, and is what families and others support with their donations.

Work is underway on the exciting agenda for the 24th annual Scientific Symposium, scheduled for Sept. 27-30, in Denver, Colo. ■



Christopher Mathew, David Frohnmayer, Hans Joenje and Juan Bueren

"I would especially like to thank the FA patients and families who have been so generous in supporting our work and providing precious samples...God bless the good ship FARF and all who sail in her, and may our collective efforts yield the therapies for which there is such great need."

—Christopher Mathew, PhD, in his award acceptance speech



Testing Service for FA Patients

Testing for Potentially Beneficial Cancer Therapy

The Knight Diagnostic Laboratories at Oregon Health & Science University have recently made available new molecular tumor tissue tests designed to identify potential treatment targets in cancer and to predict the likelihood of benefit for patients treated with the latest therapeutics.

This new testing is available at NO CHARGE to FA patients.

For more information, contact:

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Second International FA Gene Therapy Working Group Meeting Highlights Progress



Jakub Tolar, MD, PhD, University of Minnesota, Minneapolis, chaired the second international working group meeting on gene therapy on Oct. 20, 2011, in Barcelona, Spain. Twenty-three attendees, including gene therapy and Fanconi anemia experts from seven different

countries, participated. Inspiration for these meetings arose from gene therapy successes in other illnesses, the need for collaboration given the rarity of FA, and the conviction that gene therapy trials could be expedited by a multi-center approach. This successful meeting

highlighted tremendous progress since the first meeting in November 2010. Participants confirmed the benefits of collaboration in making gene therapy a therapeutic reality for FA patients.

Scientists engaged in a lively discussion concerning unresolved issues. Questions remain about how best to mobilize a sufficient number of stem cells for gene correction, on the need and type of conditioning necessary, and which vector will be optimal. Gene therapy trials could clarify these issues, and several groups reported that they hoped to open a trial soon (see related story below).

Dr. Tolar said that gene therapy has gone “from a concept to a reality.” He noted a “fresh sense of possibility” and the value of building on a tested model. He concluded: “We are close to solid action. This will happen!” ■

Experts Discuss Progress of Gene Therapy Efforts

At the October 2011 Fanconi anemia gene therapy working group meeting, gene therapy experts updated their progress in making gene therapy a reality for FA patients.

Pamela Becker, MD, PhD, Institute for Stem Cell and Regenerative Medicine, University of Washington, and Hans-Peter Kiem, MD, Fred Hutchinson Cancer Research Center (FHCRC), both Seattle, Wash., have initiated a gene therapy trial at the FHCRC, initially for three FA-A adults. This trial will use a lentiviral vector developed in Dr. Kiem’s lab for gene correction, and will initially not use conditioning prior to infusion of gene-modified cells. **This trial has recently received FDA approval and is now enrolling patients.**

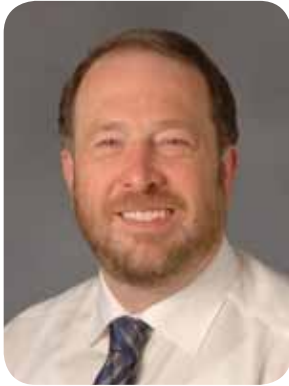
Dr. Kiem speculated that gene correction followed by expansion of corrected stem cells in the laboratory could address the problem of low stem cell counts in FA patients. He presented some encouraging data for hematopoietic stem cell expansion using a variety of different strategies.

Juan Bueren, PhD, CIEMAT/CIBERER, Madrid, plans to conduct a gene therapy trial with FA-A patients

who are part of the Spanish Fanconi Anemia Network. He and his collaborators have received funding from the Ministry of Health to collect hematopoietic stem cells from 10 patients, and will administer lentiviral-mediated gene therapy to five patients. They plan to mobilize and collect stem cells by the end of 2012; patients will be recruited for the gene therapy trial in 2013. The first two patients will receive no conditioning. If there is no engraftment, subsequent patients might receive conditioning.

Wade Clapp, MD, and Helmut Hanenberg, MD, Indiana University School of Medicine, Indianapolis, are developing a novel envelope for lentiviral vectors. They have corrected the hematopoietic system in two FA mouse models (*Fanca* and *Fance*) and achieved very high gene transfer efficiencies into normal human hematopoietic stem cells. If necessary, they will use conditioning methods that do not cause DNA damage. In 2012, they will apply to the FDA to conduct a trial with FA-A patients. This trial will initially be conducted in Indianapolis, but eventually will include other centers such as Cincinnati Children’s Hospital Medical Center. ■

Danazol Improves Blood Counts in FA Patients



Stem cell transplant is not always an option for Fanconi anemia patients, and others choose to delay going to transplant. To stabilize or improve blood counts, many FA patients have taken oxymethalone, a synthetic androgen. However, this drug has

severe and often irreversible masculinizing side effects, which are unacceptable for many patients, especially females.

In recent years, some FA patients have turned to danazol, a synthetic androgen derivative, to improve blood counts. In a 2011 article for *Blood Cells, Molecules and Diseases*, Helmut Hanenberg, MD, Indiana University School of Medicine, Indianapolis, published a retrospective report on eight FA patients who received danazol for the off-label treatment of marrow failure.

At the beginning of treatment, patients had already experienced severe marrow failure, with an average hemoglobin count of less than 8 g/dL and/or less than 30,000 platelets/ μ L. The starting dose of danazol was approximately 5 mg/kg body weight/day.

Seven out of eight patients had a robust response to this therapy. Within six months, platelets and hemoglobin increased more than 50% from the starting counts and remained stable for up to three years, despite a reduction in the danazol dose. Platelet counts in four patients who received treatment for more than three

years increased to an average of 68,000, a 2.8 fold increase over the starting value. Hemoglobin stabilized at greater than 11 g/dL. White cell counts were not significantly affected by danazol.

One patient with severe congenital abnormalities did not respond to danazol or to oxymethalone. One patient had an excellent response to danazol for more than three years, but then developed myelodysplasia (MDS) while on this therapy and died of chronic GvHD at age 26, following an unrelated donor transplant. None of the other patients developed MDS while on danazol. There were no short- or long-term toxicities associated with danazol that led to discontinuation of therapy.

Seven out of eight patients had a robust response to danazol.

Dr. Hanenberg noted that the seven patients who responded well to danazol had few or no congenital abnormalities and experienced bone marrow failure at a later age than most FA patients. He suggests the possibility that danazol may be more effective in patients with a milder phenotype.

Dr. Hanenberg very much welcomes any information about other patients' experiences using danazol so that his planned international study can benefit from additional case examples. He can be contacted at: hhanenbe@iupui.edu. ■

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.

For more information, contact:

Teresa Kennedy, Fanconi Anemia Research Fund, Inc.
Phone: 541-687- 4658 or Email: teresa@fanconi.org



Fanconi Anemia
RESEARCH FUND, INC.



**Working together to
advance FA research.**

Transplantation Outcomes for FA Patients with Leukemia and Advanced Myelodysplasia



Margaret MacMillan, MD, University of Minnesota, Minneapolis, reported on the outcomes of hematopoietic cell transplantation (HCT) at her institution from 1988-2009 for Fanconi anemia patients with acute myeloid leukemia (AML), lymphoblastic leukemia

(ALL) or advanced myelodysplastic syndrome (MDS). A total of 21 FA patients with AML (n=16), ALL (n=2) or advanced MDS with greater than 5% blasts (n=3) underwent HCT. Five of these patients had mutations in *BRCA2*. For the entire cohort, median age was 15.5 years, but median age for *BRCA2* patients was substantially lower at 1.9 years. *BRCA2* patients develop acute leukemia at a much younger age than those from other complementation groups.

Most patients had alternative donors (n=18), but three had HLA-matched sibling donors. The preparatory

regimen included either total body irradiation or busulfan. Ten patients were transplanted before the use of fludarabine (FLU); eleven received FLU.

The probability of neutrophil engraftment was 75% without FLU and 100% with FLU-based regimens. The probability of acute GvHD was 19%.

For the entire cohort, survival at one year was 43%. Survival was higher in the 11 patients who received a FLU-based regimen (50%) and in the five patients with *BRCA2* mutations (60%). For the entire cohort, probability of relapse was 19%.

These data suggest that HCT with a FLU-based preparative regimen offers a potential cure for acute leukemia or advanced MDS in FA patients. **It is important to differentiate between patients with early MDS and those with advanced MDS with greater than 5% blasts. Outcomes in patients with early MDS are much more favorable and are not included in this analysis.**

The University of Minnesota is now evaluating the effectiveness of pre-transplant chemotherapy for patients with advanced hematopoietic disease. ■

Cancer Recurrence in FA: Therapeutic Implications



The incidence of head and neck squamous cell carcinoma (HNSCC) is many times higher in Fanconi anemia patients than in the general population. Cancer stem cells (CSCs), which are resistant to therapy and drive tumor recurrence, are

present in both FA and sporadic HNSCCs.

Dr. Ian Mackenzie, DDS, PhD, Barts and The London Medical School, London, UK, stated that CSCs consist of two distinct types of cells. Some form tightly packed colonies of non-motile cells; others are highly motile, spindle-shaped cells that express epithelial-mesenchymal

transition (EMT) markers. EMT within the CSC population has been implicated in local tumor invasion and distant metastasis.

Tumor necrosis factor alpha, which is over-expressed in FA cells, enhances EMT and cells lacking the normal *FANCA* or *FANCC* gene have higher levels of these highly motile cells. Dr. Mackenzie suggests that this finding may explain the high level of recurrence of FA cancers.

Selective targeting of CSCs is of special therapeutic value for FA patients, given the severe side effects of DNA-damaging chemotherapies and radiation. Of particular interest, motile and non-motile cancer cells have different responses to cancer therapies. This suggests the strong need to develop combinational therapies that selectively kill both kinds of cancer cells. ■

Induced Pluripotent Stem Cells: The Hope and the Challenge



The Spring 2010 FA Family Newsletter reported on the potential of induced pluripotent stem cells (iPSC) to correct hematopoietic cells in Fanconi anemia patients. Scientists hope to remove skin or bone marrow cells from a patient, correct the genetic defect and reprogram these cells to

become iPSCs. The corrected cells would be capable of self-renewal and could differentiate into other cell types, including blood-producing cells.

A keynote address by Suneet Agarwal, MD, PhD, Children's Hospital Boston, at the Scientific Symposium, and a panel discussion by experts described the many challenges that must be overcome before this technology can be available for patient therapy.

FA cells themselves present a barrier to reprogramming. Some strategies to reprogram cells activate DNA damage, and damage repair is faulty in FA cells. Methods that utilize non-integrating strategies hold potential. Yet the multi-step process required by this technology can create carcinogenic mutations. Present techniques for causing iPSCs to differentiate are not very effective.

In addition, scientists still cannot isolate the true hematopoietic progenitor cell from iPSC, which presents a significant hurdle for the effectiveness of iPSC methodology. Obtaining large numbers of disease-free hematopoietic cells will be challenging, and scientists don't yet know if blood-producing cells created by iPSC technology are truly functional.

Although iPSC technology holds promise for FA patients, symposium participants candidly warned that many barriers must be overcome before this technique is efficient, effective and safe for patients. ■

Inability to Detoxify Aldehydes May Contribute To FA Phenotype

Aldehydes are common carcinogens. They are produced within organisms as by-products of cellular metabolism, are present in the environment (an example is formaldehyde) and are formed by oxidation of alcohol. Aldehydes are broken down or detoxified through the action of special enzymes. The inability to detoxify aldehydes may be a fundamental defect in Fanconi anemia patients, according to recent important findings.

K.J. Patel, MD, PhD, MRC Laboratory of Molecular Biology, Cambridge, UK, and his collaborators demonstrated that aldehyde exposure combined with an inability to break down aldehydes is extraordinarily toxic in *Fancd2*-deficient mice (*Nature* 475:53-58, 2011). Normally, these mice do not have physical anomalies associated with FA and do not develop spontaneous aplastic anemia or leukemia. However, *Fancd2*-deficient mice bred to lack an enzyme that detoxifies aldehydes have characteristics seen in FA patients. Maternal alcohol exposure led to skeletal defects in these mice, and alcohol

consumption after birth rapidly precipitated bone marrow failure. These mice also spontaneously developed acute leukemia.

Dr. Patel speculates that the developmental defects, bone marrow failure and cancer predisposition that characterize individuals with FA are caused by DNA

This important study suggests new therapeutic approaches to treat FA.

damage resulting from an inability to break down aldehydes. Aldehyde accumulation is particularly toxic to FA patient bone marrow cells.

This important study suggests new therapeutic approaches to treat FA. For example, it might be possible to induce the detoxification of aldehydes by treating patients with a small molecule such as Alda-1. This small molecule activates enzymes that break down aldehydes. ■

Alda-1: A Possible Therapy for Fanconi Anemia



The finding of K.J. Patel (see article on page 6) that aldehyde accumulation contributes to the development of the Fanconi anemia phenotype in *Fancd2*-deficient mice suggests a possible therapy for FA patients, according to Daria Mochly-Rosen, PhD, Stanford University

School of Medicine, Stanford, Calif. Dr. Patel's research suggests that aldehydes are especially toxic to FA cells and contribute to the bone marrow failure and cancers that characterize this disease. Increasing a patient's capacity to detoxify aldehydes might prove highly beneficial.

Dr. Mochly-Rosen and her collaborators developed a

small molecule called Alda-1 that activates an enzyme that detoxifies or breaks down aldehyde. To test the effectiveness of Alda-1, mice were administered 3.33 g/kg of alcohol, an amount equivalent to binge drinking

Research suggests that aldehydes are especially toxic to FA cells and contribute to bone marrow failure and cancers.

in humans. Twenty minutes after ingestion of alcohol, mice treated with Alda-1 had dropped their blood acetaldehyde levels by 40% compared to a control group.

Because aldehyde toxicity may contribute to the diverse complications that characterize FA, the use of Alda-1 as a treatment in models of FA is highly warranted. ■

Human Papillomavirus and Head and Neck Cancer



Maura Gillison, MD, PhD, The Ohio State University Comprehensive Cancer Center, Columbus, presented a keynote address on the human papillomavirus (HPV) and head and neck cancer in the general, non-Fanconi anemia population.

Dr. Gillison reported that the percentage of HPV-positive head and neck cancers has increased dramatically in recent years, from 16% in 1984 to 72% by 2004. These cancers are found primarily in the oropharynx, an area including the base of the tongue, tonsils, the back of the mouth and the middle part of the throat, but are also found in the oral cavity.

Sexual contact is the primary risk factor associated with HPV-positive head and neck cancers. Risk increases with the number of sex partners, the number of oral sex partners, and history of sexually transmitted disease. Dr.

Gillison noted that HPV-positive head and neck cancer in men is more common than HPV-caused cervical cancer in women.

HPV positivity is associated with younger, non-smoking patients. Prognosis is far better than in HPV-negative patients, where smoking often plays a large

Dr. Gillison believes that HPV vaccines will be useful in decreasing head and neck HPV-positive cancers.

causative role. HPV-positive tumors are more responsive to radiation and chemotherapies. Erbitux is often effective in treating these tumors. In contrast, smoking reduces the response to therapy.

Dr. Gillison believes that HPV vaccines will be useful in decreasing head and neck HPV-positive cancers, but this hypothesis has not been studied. HPV vaccines were developed to prevent cervical cancer in women. Researchers were not then focusing on the rapidly growing number of HPV-positive head and neck cancers. ■

Study Suggests Possible Role of HPV in FA Cancers



Fanconi anemia patients are at high risk of developing squamous cell carcinoma of the head and neck and anogenital tract. In the general population, these tumors frequently express the high-risk human papillomavirus (HPV) subtype 16 and its cancer-causing gene *E7*.

Susanne Wells, PhD, Cincinnati Children's Hospital, is researching the possible role of *HPV16E7* in causing some of the cancers that affect FA patients.

Dr. Wells conducted experiments to determine if the loss of function of an FA gene increases the likelihood that exposure to HPV would lead to cancer. She transduced human skin cells with the cancer-causing gene *HPV16E7* and studied the effect of the loss of *FANCA* and *FANCD2* on these skin cells. Dr. Wells showed that loss of FA genes stimulated accumulation of the E7 cancer-causing protein, leading to epithelial

and basal cell proliferation, diagnostic features of HPV-related cancer.

Dr. Wells' findings suggest that individuals with FA are either more susceptible to new HPV infections or prone to viral persistence once infected.

If HPV infection occurs in a young child with FA, early HPV vaccination would be warranted.

If HPV infection occurs in a young child with FA, early HPV vaccination would be warranted. However, if HPV infection occurs *in utero* or at the time of birth, vaccination may not be effective against the particular HPV subtype causing the infection.

A previous study by Cincinnati researchers determined that some FA patients are HPV positive as young as ages 6 and 7. Dr. Wells underscored the critical importance of determining the mode of HPV transmission in young children. ■

Use of Antioxidants to Reduce Chromosomal Breakage



Filipa Ponte, PhD student, University of Ponto, Portugal, said that Fanconi anemia characteristics including bone marrow failure and malignancy may be related to an inability to prevent oxidative stress and repair DNA damage. Her research has focused

on evaluating two antioxidants, α -lipoic acid (α -LA) and N-acetylcysteine (NAC), for their capacity to reduce chromosome breaks in lymphocytes from FA patients.

Ponte evaluated both antioxidants individually and in a cocktail combining the two antioxidants. When tested

alone, each antioxidant significantly reduced the number of chromosomal breaks in FA lymphocytes. Combining the antioxidants resulted in a 60%-80% reduction in chromosomal breaks compared to a lower effect with the use of α -lipoic acid and NAC alone.

Each antioxidant significantly reduced the number of chromosomal breaks in FA lymphocytes.

Ponte suggests that α -LA plus NAC can be an effective antioxidant cocktail for FA patients, to delay or possibly prevent bone marrow failure and early cancer development. ■

Do FA Patients Require Higher Dosages of HPV Vaccines to Achieve Protection?



HPV vaccines might prevent some of the cancers that affect Fanconi anemia patients, but it is critical for the patient to mount an effective immune response to these vaccines. Jason Taylor, MD, PhD, Oregon Health & Science University, Portland, Ore., hypothesized that

immunological defects in FA might make it difficult for these patients to develop sufficient antibodies in response to the HPV vaccines.

Dr. Taylor analyzed bone marrow from five FA patients and determined that these samples had low levels of B-cells (less than 10% of normal). He further confirmed altered levels of B-cell activation in the bone marrow of *Fancc*-deficient mice.

Using a dose that mimicked the human vaccine, Dr. Taylor injected the FDA-approved HPV vaccine, Cervarix, into normal and *Fancc*-deficient mice and

compared their responses in mounting antibodies. Normal mice had a higher antibody response compared to *Fancc*-deficient mice. A five-fold larger dose of both approved vaccines, Gardasil and Cervarix, produced a higher antibody response in both *Fancc*-deficient and normal mice. Cervarix elicited a stronger response than Gardasil in both groups of mice.

Immunological defects in FA might make it difficult for patients to develop sufficient antibodies in response to the HPV vaccines.

These preliminary mouse studies suggest that FA patients may require a higher vaccination dosage in order to obtain adequate vaccine efficacy against HPV. Further research in this area is necessary. However, the immune system of mice is not identical to that of humans. Thus, a crucial next step is to study HPV antibody response in vaccinated FA patients. ■

Two New Drugs Hold Potential for FA Patients



Eighty percent of Fanconi anemia patients develop bone marrow failure and experience a high incidence of evolution to myelodysplasia and/or leukemia. In recent years, numerous studies have linked these abnormalities to the findings that FA patients overproduce

tumor necrosis factor alpha (TNF α), which is especially harmful to FA hematopoietic cells due to their inherent hypersensitivity to TNF α . Recent research demonstrates that several drugs known as kinase inhibitors can neutralize the overproduction of TNF α .

Johanna Svahn, MD, G. Gaslini Children's Hospital, Genoa, Italy, reported on her studies of monocytes isolated from peripheral blood from six FA-A patients. The FA patients' monocytes overproduced TNF α compared to normal controls. Dr. Svahn and co-workers found that two drugs, BIRB796 and dasatinib, significantly reduced TNF α in monocytes from these patients. Both drugs are in clinical trials for other disorders.

If preclinical studies demonstrate an *in vitro* beneficial effect on hematopoietic progenitor colony growth, these drugs would be candidates for testing in clinical trials seeking to enhance hematopoiesis and suppress clonal evolution in FA patients. ■

Hematopoietic Failure Begins Prenatally in *Fancc* Mice



A common assumption is that Fanconi anemia blood stem cell progenitors are normal at birth and begin to die prematurely after birth. Patient blood counts are usually normal at birth; 7 is the median age for onset of hematologic symptoms in FA patients.

Ashley Kamimae-Lanning, graduate student, Oregon Health & Science University, Portland, Ore., hypothesized that stem cell deficiency might begin prenatally. She tested this hypothesis in a *Fancc*-deficient mouse model.

Researchers studied hematopoiesis in the livers of *Fancc*-deficient fetal mice 14.5 days after fertilization. These mice have a 10% reduction in body mass and 33% lower total liver cell count. Their livers contained

significantly fewer hematopoietic stem and progenitor cells compared to their normal littermates. *Fancc*-deficient fetal liver hematopoietic stem cells also demonstrated decreased transplantation capacity, suggesting premature stem cell exhaustion.

Mouse studies suggest that hematopoietic failure begins prenatally and undermine the conventional belief that stem cell depletion begins after birth.

These mouse studies suggest that hematopoietic failure begins prenatally and undermine the conventional belief that stem cell depletion begins after birth, at least in mice. ■

Fanccp-deficient Mice Develop Cancers Seen in Fanconi Anemia Patients

The most recently identified Fanconi anemia gene is *FANCP*, also known as *SLX4*. Gerry Crossan, graduate student, MRC Laboratory of Molecular Biology, Cambridge, UK, announced that his lab has generated and characterized a *Fanccp*-deficient mouse. Unlike mouse models of other FA complementation groups, the *Fanccp* model shares many features of FA in humans. These mice frequently display developmental defects, and a small number develop bone marrow dysfunction. The majority of older *Fanccp*-deficient mice develop cancer. Predominant cancers are leukemia, liver cancer and rectal carcinoma, cancers known to occur in FA patients. This mouse model mirrors both the short-term features and long-term complications associated with human FA.

One of the biggest limitations in FA research is the lack of proper animal models of the human disease. Few FA mice have characteristics similar to FA in humans.

The *Fanccp* mouse model promises to be an excellent model of human disease, and can be used to test therapeutic interventions.

The *Fanccp* mouse model, prone to epithelial cancers, promises to be an excellent model of human disease, and can be used to test therapeutic interventions. ■

Since 1989, the Fanconi Anemia Research Fund has awarded **175** research grants to **92** researchers at **51** institutions totaling **\$14.1 million**. For details, go to www.fanconi.org.

Researchers Make Progress in Developing New Small Molecule Therapies for Fanconi Anemia

The National Heart, Lung and Blood Institute of the National Institutes of Health funded a large project to develop small molecule therapies for the treatment of Fanconi anemia. This program, initiated with grants from the Fanconi Anemia Research Fund, has now entered its second year. Following is an update from each of the three institutions involved in the project, under the overall direction of Markus Grompe, MD, Oregon Health & Science University (OHSU), Portland, Ore.:

- Alan D’Andrea, MD, Harvard University, Boston, is using high throughput screening of FA cells in a Petri dish to find molecules that improve the survival of FA cells.
- John Postlethwait, PhD, University of Oregon, Eugene, Ore., studies zebrafish with FA and tests drugs that mitigate DNA damage.
- Grover Bagby, MD, OHSU, uses mice and human cells to unravel the effects of cytokines on FA bone marrow. His laboratory is working on drugs to reduce the production of TNF-alpha, which is overproduced in patients with FA and thought to play a role in bone marrow failure.
- Dr. Grompe’s group has produced induced pluripotent stem cells from FA patients that can be used to screen potential new drugs in the laboratory.

In addition, his lab is testing compounds in FA mice to determine whether these substances can delay cancer and/or bone marrow failure.

In the last year, all projects have identified new compounds that improve key properties in FA cells, FA fish or FA mice. Many of these drugs have never been used before in FA and must be validated in additional experiments. Nonetheless, the team is very excited about the new leads for therapy.

All projects have identified new compounds that improve key properties in FA cells, FA fish or FA mice.

Studies with FA mice show that certain antioxidants, e.g., tempol, can delay tumors; other drugs, e.g., resveratrol, can improve bone marrow function, but the doses required are too high to be practical in human patients. However, similar compounds which can be given orally in clinically relevant doses are now showing promise in mouse experiments. If fully validated in currently pending animal studies, clinical trials for these medicines will be planned. ■

**24th ANNUAL
Fanconi Anemia Research Fund
SCIENTIFIC SYMPOSIUM**



**SAVE
THE DATE**

September 27-30, 2012
Grand Hyatt Hotel

**DENVER
COLORADO**

Two Young Adults Chosen to Represent FA Perspective at the National Level



Amy Frohnmayer and Christopher Byrd, two young adults with Fanconi anemia, were selected last fall to serve in the Patient Representative Program, a new initiative of the US Food and Drug Administration (FDA). This initiative gives patients a voice in medical product development and regulation processes. Amy and Christopher will represent patients with orphan diseases, and will serve on advisory committee panels and other FDA-related workgroups.

Amy, 25, a research assistant at Oregon Health & Science University, Portland, Ore., and Christopher, 29, an environmental lawyer for the state of Florida, completed a multi-step process to become Special

Government Employees, a required designation. They now have graduated from an “FDA 101” training course, and are ready to participate as patient representatives.

“It’s an honor to have been accepted as an FDA Patient and I’m eager to participate and represent the FA family to the best of my abilities,” said Christopher. Amy agreed, saying, “I’m grateful for this opportunity and simply hopeful that I can contribute in a way that supports the needs of our community.”

Amy and Christopher will represent patients with orphan diseases, and will serve on advisory committee panels.

Both Amy and Christopher were chosen because of their leadership skills, their ability to speak out on complex medical issues, and their willingness to confront and challenge obstacles in our research and treatment systems. Their professional expertise and personal experience with FA make them ideal representatives to this program. Congratulations, Amy and Christopher! ■

New 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 new research study funded by FARF to determine if saliva can be an early detection tool for oral cancer. Contact Teresa Kennedy as soon as possible after diagnosis and before treatment at teresa@fanconi.org or 888-FANCONI. Teresa will coordinate your participation with David Wong, DMD, DMSc, the study’s principal investigator. For more information, visit Research Highlights on our website.

SCC Fact Sheets Available

Regular screenings for oral cancer are critically important for FA patients. The Fund has prepared helpful fact sheets about squamous cell carcinoma to share with your dentist and ear, nose and throat doctor (ENT). **We recommend that you take a fact sheet to every dentist and ENT visit.** For copies of the fact sheets—available in English, Spanish, Afrikaans, Dutch, French, German, Hebrew and Italian—contact our office or visit the Publications section on our website.

FA One Moment at a Time: Steps in Coping

By Nancy Cincotta, MSW, MPhil, Psychosocial Director, Camp Sunshine

Living and dealing with Fanconi anemia comes with unique complications. Your world is turned upside down at diagnosis, whether it is unexpected or comes after a long process of medical discovery. From the day you learn that you have become a member of this group, one you never would have chosen, you strive to come to terms and live (even thrive) with the diagnosis.

Often we learn to cope through collective wisdom. FA challenges that approach, as FA's wisdom is not generations old and the expectations regarding its course are ever-changing, so espousing and utilizing collective wisdom is difficult. What is the emotional lesson to be learned? Understand where you are, understand where FA research and treatment are, and maintain hope and flexibility, appreciating that the knowledge base and management of FA evolves constantly.

Living with the certainty of uncertainty is its own challenge. Can you occasionally put FA in the back of your mind to manage the day ahead? Or does it need to be in the forefront of your mind, with the only relief being sleep, if you do sleep? People differ on the issue of "partializing," i.e., emphasizing parts, rather than the whole, of FA. These differences can be embraced, as people with different coping styles can help and complement each other.

The terrain for each child, teen or adult with FA will always be somewhat uncharted. Whether a parent, a sibling or someone diagnosed with FA, at the end of the day, you have to live with yourself and feel that you can turn off the light and find peace in your place in the fight. ■

Some Potential Strategies for Coping

Information

- Learn as much as you can about FA and its treatment. Information will increase your understanding and help you cope.
- Stay abreast of evolving knowledge.
- Help advance science in some way—participate in a study; fundraise.
- Be proactive in the face of the illness.

On your personal journey

- Partialize the issues you're facing.
- Learn who you are and what helps you feel your best.
- Understand your coping style and nurture it.
- Be kind to yourself.
- Develop long-term goals while making short-term plans.
- Enjoy and treasure simple moments.
- Seek joy, a sense of accomplishment and meaning in every day, even on bad days.
- Value successes, large or small.
- Acknowledge and value the strengths of all your family members.
- Let your kids be kids, whether you are the parent of someone with FA, or you have FA and have children.
- Respect your privacy and the decisions you make. You and your family are the only ones who can ultimately make your important medical decisions.
- Grieve your losses, large and small.
- Look for connections, for people you trust, for people who understand.
- Find hope. It can be cloaked in many different ways.

Collective Wisdom

- Engage in the use of collective wisdom from the FAfamily.
- Seek refuge. Find someone in the FAfamily to use for support.
- Find meaning. Help yourself, help other FA families, and help the Fanconi Anemia Research Fund.
- Use the annual Family Meeting at Camp Sunshine and the listserv as resources for support and group wisdom.
- Be connected: do something for FA Day on May 1.
- Embrace what FA research has enabled. Understand that you can move it further.
- Use the gift of forethought and dedication of the Frohnmayer family. Meet them and those active in FARE. Use them for inspiration and dialogue on this journey.

Global Family Support Initiatives Gain Momentum



Families gather in Barcelona.

FA Families Initiate Even Greater Collaboration

Families from France, the United Kingdom, Italy, Spain, Germany, Belgium, The Netherlands and the US held a spontaneous and helpful meeting in conjunction with the Fanconi Anemia Research Fund’s annual Scientific Symposium in Barcelona, Spain, last October. The families, representing FA support groups in their respective countries, discussed their many services and activities, and how the groups can work even more collaboratively. They explored how to attract European Union (EU) funding, develop an EU-wide registry, and how to use the early cancer detection equipment and facilities available through the German group. This exciting first step has helped to further unify the FA family throughout Europe and the US!

Third Brazil FA Family Meeting

The Third Brazil Fanconi Anemia Family Meeting was held in November in Curitiba, Brazil. The annual event is organized by Carmem Bonfim, MD, Hospital de Clinicas Pediatric Stem Cell Transplant Program and FA Outpatient Clinic. Dr. Bonfim enthusiastically reports that 93 FA patients attended. Eunike Velleuer, MD, and Ralf Dietrich, German FA Support Group, traveled from Germany and provided oral examinations for 90 patients. Also during the meeting, families decided to create a Brazilian FA family support group to be led by FA parent Francys Vilella.

Spanish FA Family Support Group Meets in Barcelona

The Spanish Fanconi Anemia Family Support Group held a meeting, organized by Jordi Surralles, PhD, Universitat Autònoma de Barcelona, prior to the start of the Fund’s Scientific Symposium. Dave and Lynn Frohnmayer, co-founders of the Fanconi Anemia Research Fund, introduced themselves and talked about the history of the Fund. Teresa Kennedy, the Fund’s Director of Family Support Services, spoke about the services the Fund provides to families worldwide. In total, 23 Spanish families attended the meeting. Nine FA clinicians also participated, including Jakub Tolar, MD, PhD, University of Minnesota, who answered families’ questions on a variety of FA topics. Following the meeting, six of the families registered with the Fund.



Fanconi Anaemia South Africa is Formed; Website Launched

Wynand van der Merwe has started a support group in South Africa for families affected by Fanconi anemia in memory of his wife, Madeleine, who died in 2011 at age 28 from complications related to cancer treatments. Visit the group’s website, which was created to raise awareness, and to inspire and motivate others to help people affected by FA, at <http://fanconi.co.za/>. Wynand recently applied for nonprofit status for the organization with a goal of providing financial aid to people living with FA in need of medical care and of raising awareness of this rare disease.

Find us on Facebook at 
www.facebook.com/fanconianemiaresearchfund

First Israeli Family Gathering Held in Tel-Aviv

By Sharon Harari



Alma, Sharon, Yavin and Amitai with Johnson the dog

The delicious food was left untouched. Earlier that day, as I was thoughtfully picking what seemed to be the most appetizing kosher pastries at the local bakery, my thoughts were focused on one thing: *We need comfort food for tonight.*

That night, Nov. 19, 17 Israeli Family members gathered in a small apartment in Tel Aviv for the first time. My husband Yavin and I had arrived early and set the chairs in a circle in my mother's apartment, thinking that is what Nancy Cincotta, the psychosocial director at Camp Sunshine, would have done if she were with us. But Nancy wasn't there; we were on our own, ready to launch this new group with a deep sense of responsibility and a hope that a successful evening would prove our efforts to be worthwhile.

At 8 pm the doorbell rang and for the next hour a constant stream of people came in. Most of us had never met before, some had spoken over the phone or

corresponded by email, for others this was the first time they would meet another family or patient with FA.

We were a small crowd, but quite diverse. There were Orthodox and secular Jews, people from the northern part of the country and from the south, young couples with children recently diagnosed and older folks who had been living with FA for many years. Two young adults drew everyone's attention immediately—a 24-year-old man and a 30-year-old woman, both with FA.

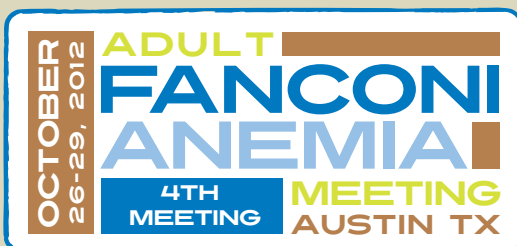
It was an emotional evening, and if I weren't confident that the pros would outweigh the cons, I would have never pursued the idea of bringing us all together in one room in the first place. For some, it was an opportunity to talk about things they had never spoken out loud about, for others it was a time to learn from someone else's experience and listen to good advice.

The stories were powerful and moving, overwhelming at times and sad, but words came straight from the heart and there was a lot of nodding.

A fellow FA mother, a practicing physician, helped translate and edit what we felt was essential information that was later handed out along with a small family directory so we could all stay in touch.

After four long hours of talking and listening we were hugging and saying our goodbyes. All of us were still struggling with our personal challenges, but this time equipped with a new perspective, perhaps a few more tools in our mental toolbox and 16 new compassionate friends.

As the last guest left the apartment I gathered all the leftovers and smiled at the thought of my urge to comfort others with pastries. The fine cuisine was really unnecessary. It was the mental food and support that this group of people gave each other that really mattered. ■



Meeting for Adults with FA

Oct. 26-29 • Austin, Texas

Questions or need financial assistance for travel? Please contact Teresa Kennedy at teresa@fanconi.org or 1-888-FANCONI.

Parent Helps Bring FA To British Television

By Jeannie Dalglish



Robert, Louise, Jeannie and Alex; *Emmerdale* cast members

My husband Robert and I have a son, Alex, 16, and a daughter, Louise, 11. Louise was diagnosed with Fanconi anemia at age 7, when we took her to see a pediatrician because of severe bruising. We were lucky to meet a wonderful consultant, Dr. Mary Morgan, who had experience with FA patients and offered us an incredible amount of support. At that time, it was not possible to test for the complementation group in the UK, so we went to Germany. There we met Dr. Helmut Hanenberg who was able to do these tests and discuss the treatment. Later, we met members of the German support group led by Ralf Dietrich. With their encouragement, Robert, along with Thomas Carroll, another parent of a child with FA, started the British charity, Fanconi Hope Charitable Trust. The organization has the support of three clinicians as trustees and the Duchess of Devonshire as patron.

At first Louise didn't need any treatment, but as her blood counts started to fall we decided to try danazol. We were obviously concerned about the side effects. After six months her counts rallied and then stabilized. She started on 100mg twice a day and we did see some side effects, but nothing serious. However, we reduced the dose to

100mg every day and saw no further side effects and, importantly, her improved blood counts continued.

Aware that the steroid treatment doesn't always continue to work, and that Louise only had one matched donor on the registry, we decided to look into bone marrow transplants. Louise was given a large number of tests to ascertain how her body was coping. Her bone marrow cellularity has increased markedly since starting on danazol and now stays at around 60%, so the general consensus is that she should continue on danazol for the foreseeable future.

I see from the FA family egroup that some of you have managed to see *Emmerdale*, a popular series in the UK. I was first approached in March 2011 by someone at ITV proposing that they introduce FA into the *Emmerdale* storyline. This was clearly an amazing opportunity to help to raise the profile of FA.

I have been very impressed by the tenacity of the series' researchers. We have had many conversations to help them understand the condition. They even read the UK FA Standards of Care! I provided the names of various clinicians, nurses and organizations that I felt could best answer their questions about the care of patients in the UK. After the on-screen "diagnosis," visits to the Fanconi Hope website soared to around 40 times the normal level.

Louise has loved watching the program and visiting the set. She recently received a birthday card from the actress who plays the FA mother, and will meet her soon. Naturally she is very excited.

We were delighted to be able to meet some of the members of the Fanconi Anemia Research Fund at their Scientific Symposium in Barcelona and also to meet parents from some of the other European support groups.

When Louise is able to fly again, we hope to make it to Camp Sunshine and meet more of you. ■

FA FAMILY MEETING AT CAMP SUNSHINE



**Casco, Maine
Aug. 10-15**

Questions or need financial assistance for travel? Please contact Teresa Kennedy at teresa@fanconi.org or 1-888-FANCONI.

Lessons Learned From My Journey with FA

By Beth Evans



Beth, Abreham, Eyasu and Matt

My journey with Fanconi anemia began when I was diagnosed at the age of 5. My brother, Barry, who was seven years older than I, had been diagnosed a few years before at the age of 10. Barry and I were perfect HLA matches, but, of course, we couldn't help each other. My two other brothers were not matches. Unrelated transplants had a very low success rate two decades ago. Barry died in 1988, at the age of 20, from bone marrow failure.

I only had minor complications growing up, such as moderately low counts and frequent infections, some requiring hospitalizations. I graduated high school, went to college and got married in 2001. In 2005, I found out that I was going through early menopause at the age of 30 because of FA, which meant that we would be unable to have biological children. So we began to look into adoption.

In preparation for a new family, we decided to find a physician who had experience with FA adults just in case something catastrophic happened. Our research led us to Dr. Farid Boulad at Memorial Sloan Kettering Cancer Center in New York City. We traveled there in May 2009 and were very pleased with the entire facility. Dr. Boulad is the most compassionate doctor I have ever met, and an expert on FA and bone marrow transplants. At that time my counts were very good. He recommended that I have a bone marrow aspiration because it had been six years since I had one, but I put it off.

In April 2010, we adopted two boys from Ethiopia, Abreham, 7 months, and Eyasu, 3. We were so excited about beginning this new chapter in our lives. But a few months later my counts began to drop and I had several infections back to back. I finally had a bone marrow aspiration and found out that I had high grade myelodysplastic syndrome which turned into acute

myeloid leukemia within weeks. We were so grateful that we had previously seen Dr. Boulad, because we immediately knew whom to contact. Within a week, we saw Dr. Boulad and he recommended that we move to transplant. They began the search for a donor and were able to find a 10/10 match for me.

I had radiation and chemotherapy and had the transplant on Dec. 29, 2010. I was 35 years old, which meant this was a high risk transplant. I developed lung toxicity and spent a few days in the observation unit because my oxygen level was in the 70s. I got out of the hospital on Jan. 18, but had to remain in the area during my recovery. Things were going well until we found out in February that I had an active infection of Epstein-Barr virus (EBV). I began treatment for EBV with IV Retuximab. I had to have a tonsillectomy and my adenoids removed; not an easy procedure for an adult. I was unable to swallow because of EBV in my throat and GvHD so I had a feeding tube. Eventually my doctors used T-cell therapy to fight EBV. I was finally able to go home to Georgia last October. In January, laboratory tests showed I was EBV-free, and I have been able to go back to work and resume normal activity.

I have learned a few things that I would like to share:

First, find a good FA specialist and have appropriate testing done such as a yearly bone marrow aspiration and regular CBCs. Have a plan in case something catastrophic happens like MDS, leukemia or other cancers. We were so thankful we had met with Dr. Boulad and chosen a hospital where I would have a bone marrow transplant if ever needed.

Stay away from things like drugs, alcohol and smoking that can be so dangerous for those of us with FA.

Growing up with a life-threatening condition can be very difficult emotionally, so have an outlet where you can share your feelings and issues. My faith played a huge role for me. I have received the FA Family Newsletter since I was 15, but have never gone to the FA Family Meeting at Camp Sunshine. That would have been a great outlet for me, because I had never met another person with FA other than my brother. I hope to go to Camp Sunshine this summer and meet some of you! Please feel free to contact me at beth@rockbridge.cc. You can read more about my story at bethevans828.com. ■

Congratulations!



To Jacob Grossman (FA) on his Bar Mitzvah! Pictured with sister, Talia, and parents, Rachel and Alan.



To Brenda Miller (FA), pictured with husband, Mark, who celebrates her 60th birthday this month!

Survey Responses Provide Teen Perspectives on FA

The Fanconi Anemia Research Fund recently asked teens with FA from around the world to respond anonymously to questions about a variety of topics related to FA and to offer any advice they have for others. The responses were candid and thoughtful, and their wisdom has benefit for all affected by FA. Here are some excerpts. To read all the survey responses, please visit our website. Special thanks to all who participated in this survey!

What do you tell your friends about FA?

- That it's genetic and affects the bone marrow. I don't really know exactly how to explain it.
- I don't tell my friends.
- I tell my friends the truth, so that they can understand. I also tell them to look it up so that they can get more information if they want to.

If you have had a transplant, what advice would you offer to other kids who are about to have a transplant?

- My advice would be to take one day at a time. Take baby steps. Always think positively. And if you are unsure about anything you can always ask your doctors. They are there to help you.
- Keep yourself happy, don't focus on the negatives of staying in a hospital. Beyond that, expect the worst, hope for the best.
- Be prepared. It's not easy, but eventually you will feel like a normal kid again.

If you could tell your parents exactly what you need from them, what would it be?

- To stop bugging me and let me live my life.
- I would tell them that all I needed was what they were giving me already, someone to talk to when I need to and someone to pick me up when I fall, and a shoulder to cry on when I am upset or angry or scared, or just someone to complain to when life just doesn't seem fair.
- To treat me as normal.

What differences, if any, has FA made in your life?

- FA has changed my life in so many positive ways. I am able to have a better outlook on life and to appreciate it more. I am more positive. I also love to tell others about FA if they ask me.
- I just have to miss school more for more than the usual number of doctor appointments.
- What in my life hasn't changed because of FA? All my experiences with FA have made me what I am today.

What advice would you give someone just diagnosed with FA?

- Just live as you normally do. Live your life like you don't have FA. You can still have a social life; you don't have to give it up.
- I honestly have no clue.
- You will have struggles, but always try and be confident. This is who you are, so be proud of it.
- Take a deep breath, get a big hug from a parent or friend. Just keep breathing. ■

Macy's Story: Living the Happiest Life Possible

By Jennifer Stewart



Macy; Jennifer, Adam and Macy

My husband, Adam, and I welcomed our beautiful daughter, Macy, into this world on Sept. 16, 2010. She weighed 5 lbs. 9 oz. and was born with multiple congenital anomalies (hip dysphasia, a horseshoe kidney and hypoplastic thumbs). In the months following Macy's birth, numerous specialists tested her for everything related to VACTERL association. Her hip dysphasia was corrected with a palvik harness, and her horseshoe kidney was functioning well. We considered ourselves among the lucky ones, and were just thankful she was healthy.

As time went on, I couldn't help but feel that the VACTERL association diagnosis didn't quite fit. In my Internet research, I kept coming across Fanconi anemia. I would read and re-read the features of FA, and my heart sank as I realized it fit. At Macy's next genetics appointment, her doctor asked if I had any other questions. I asked if Macy should be tested for FA. His initial response was no, but later that week he decided it was best to test her in order to rule it out. Her test came back positive.

Macy was diagnosed with Fanconi anemia on May 4, 2011, at age 7½ months. We were devastated. The initial impact of the diagnosis was immense grief and fear. We knew our lives would never be the same. I immediately began to arm myself with as much knowledge of FA as I could to find the best formula that would give Macy her best chance. We decided to give her the happiest life we possibly could— full of love, positivity and lots of laughter. We couldn't see how being sad was going to help her, so we put smiles on our faces and focused on the present.

Macy is now 18 months old. She has been hospitalized for "failure to thrive," had several NG tubes, surgery to place her gastric feeding tube, and had index pollicization surgery on her left hand. She's been poked and prodded more times than I can count, but through it all she wakes up every morning with a huge grin on her face. She is an inspiration to all who know and meet her, and I am honored to be her mother.

Currently, her blood work looks great, but we know that will not always be the case. We've always wanted more children, so we have taken steps to begin a journey into *in vitro* fertilization with pre-genetic determination in order to have another child who is FA-free, and also a perfect donor match for Macy. Although we don't know what the future holds, we do know that we have an incredible little girl who is healthy right now. Her smiling face is a daily reminder of what is important in life and to make each moment count. We are now ready to face anything that comes our way, and we'll do it the best we can, one day at a time. ■

In Loving Memory

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

Karen Nazer 2/14/85 - 9/1/11

Madeleine van der Merwe 6/7/83 - 10/4/11

Celine van Heerden 11/25/96 - 2/15/12

Samuel Gillott 8/30/05 - 2/18/12

Bob Nicholson, Jr. 10/25/59 - 3/4/12

Matthew Abramov 8/1/70 - 3/23/12



Third Annual International FA Day Promises to be a Huge Success

By Peg Padden

The Third Annual International Fanconi Anemia Day is coming up May 1! As I write this, we have 23 FA families, individuals and friends doing many different, and often quite creative, things to raise awareness and money for research through the Fanconi Anemia Research Fund. Some fundraisers will take place on May 1 and others throughout the month of May, but all have the same goal—to make Fanconi anemia a disease of the past. We are motivated!

The plans so far include: Mary Jo Becerra and Tricia Robutko will each host Bunco For A Cure. The Meffords will hold their Third Annual Silent Auction, Inflatables and Turtle Races (yes, turtle races!). The Connelly, Clifton, Lackey and Ross families are all doing a letter campaign. Jeanne Atkinson will have a Knit-In and a Jewelry Party, while Kay Proctor and Nancy Cincotta will sell items on eBay. The Boggs family will contribute with their Third Annual Fun Day 4 FA; the McCarthys with their Cornhole Tournament; Matt Pearl, 15, with Kick FA; and Patti Carter and family with Cosmic Bowling. The Frohnmayers and friend Sharon Schuman will hold their 13th annual chamber

music concert and wine auction. I did mention creativity, didn't I? Well, there's more. The McQueens are holding an FA Greek Theater Party, while Victoria Hathcock will sell crocheted items. Brenda Witherspoon and Lisa Routh will have yard sales. Benjamin Morrison, 15, put on his Third Annual Chess Tournament. The Shelsons, from Toronto, will hold their annual silent auction and dinner. And last, but not least, we have Peter Fiaschetti, 12, not only planning refreshments and a bake sale at a parade, but writing to the governor of Rhode Island and President Obama requesting that May 1 be proclaimed International Fanconi Anemia Day!

International FA Day was created to raise money for research through the Fanconi Anemia Research Fund.

Last year, more than 20 families participated in FA Day and raised \$75,000 for the Fund. We were happy with that, but know we can do more. We invite other FA families, individuals and friends to join us this May and help us reach our goal. Together we will find a cure. ■

Two Scholarship Funds Support Meeting Participants

Alex Norris Memorial Scholarship Fund for Adult Meeting

Lynn and Dave Frohnmayer, co-founders of the Fanconi Anemia Research Fund, established the Alex Norris Memorial Scholarship Fund in memory of a remarkable teen who died in 1994 from complications of FA. The scholarship fund assists with travel expenses for participants of the Fund's Meeting for Adults with FA who would otherwise be unable to attend. There is a high demand for scholarship assistance to attend these meetings that provide valuable information and support to our adult community. The Fourth Meeting for Adults with FA will be held in Austin, Texas, Oct. 26-29. Please consider making a donation to the Fund, or fundraising, specifically for the Alex Norris Memorial Scholarship Fund.

KATA Foundation Seeds Family Meeting Scholarship Fund

The Kendall And Taylor Atkinson (KATA) Foundation recently contributed \$30,000 towards the Fanconi Anemia Research Fund's travel assistance scholarship program for the annual Family Meeting at Camp Sunshine. The KATA Foundation was founded by Ken and Jeanne Atkinson in memory of their children and now is additionally supported by Jack and Lisa Nash. This generous contribution helps the Fund provide travel expense assistance to eligible families who would otherwise be unable to attend the Family Meeting. Thank you, KATA Foundation! ■

Solari Charitable Trust Makes Landmark Donation



Mary Corrigan Solari

Mary Corrigan Solari is no stranger to the Fanconi Anemia Research Fund. She has given generously to the Fund every year for more than a decade in response to the annual holiday appeal letter sent by Dave and Lynn (and now Amy) Frohnmayer.

Because she's been so faithful in her support,

it wasn't a surprise when the unremarkable envelope from the Richard & Mary Solari Charitable Trust arrived in the office mail in early December. The amount of the check, however, was a breathtaking surprise: \$1,000,000! Staff members did a double-take, counted the zeros, and then gathered around a phone to call Dave and Lynn with the good news.

"Mary is a lovely woman who connected with Amy, through our letters, years ago," said Dave Frohnmayer, who met Mary and her late husband, Richard, through Dave's work with the University of Oregon. "Lynn and

I are profoundly grateful for this inspiring gift that will allow the Fund to pursue its research agenda even more aggressively."

"What you give really can make an impact on the possibility of discovering the cause of the disease. Hopefully a cure will be found."

-Mary Corrigan Solari

Mary lives an active life in Santa Cruz County in northern California. She has three daughters, eight grandchildren and three great grandchildren. In addition to the Fanconi Anemia Research Fund, Mary's philanthropic interests range from education to health care and the arts.

"What you give really can make an impact on the possibility of discovering the cause of the disease," said Mary. "Hopefully a cure will be found."

The officers of the Fund and our FA families and patients are deeply grateful to Mary Solari for her spectacular commitment to our cause. ■

8th Annual Valentine Fanconi Anemia Run/Walk Raises \$46,000 for Research



Peg Padden, organizer. Eighth year. Portland, Ore. 1,200 participants. More than 70 volunteers. 700 cinnamon rolls. \$46,000 for FA research. Incredible!

"Year after year, Peg does an amazing job in rallying the community to support FA research. Her boundless energy inspires the researchers among us to redouble our efforts. Peg simply brings out the best in people—and all of us on the starting line on those inevitably moist Sunday mornings in February."

-Peter Kurre, MD

Think Globally, Act Locally

The Fanconi Anemia Research Fund office is located on the second floor above a small retail complex. Each fall, our retail neighbors hold Customer Appreciation Days, a festive and well-attended weekend with live music, refreshments, store raffles, even free chair massages. For five years now, the retailers have chosen to donate a portion of their profits from the event to the Fund—a total of \$1,800. Our warmest thanks to our generous neighbors!



The Meridian Building, site of the Fund's office in Eugene, Ore.

FA Parents Determined to Make a Difference

Jennifer and Brian Dorman's first fundraiser was a resounding success and testament to their commitment to raise money for FA research in memory of their son, Scott. Scott, an active and popular young man who was a skateboarder, dirt biker, glass blower and bowling fanatic, died of oral cancer in 2010 at the age of 22. The Dormans held a garage sale last August that was promoted by a local newspaper and radio station. Jennifer says the years of treatment and predictions that Scott wouldn't survive to adulthood make her eager to help anyone else facing that reality. Maybe, she says, their efforts will ease another family's fight. Their garage sale effort raised \$3,050. The Dormans plan to hold a fundraiser every year to benefit FA research.

Make Lemonade

Stephanie Griggs, mother of Bella, FA, age 8, sent the Fund a donation raised by some young, enterprising and generous neighbors. Two pairs of sisters spent many days last summer selling lemonade from a front yard stand. The four girls, aged 8 to 12, pledged to donate all their proceeds to the Fund in honor of Bella. Bella's sister, AJ, helped out when she could. In addition to serving up 50-cent cups of lemonade, the girls informed their customers about FA. At the end of the season, when the entrepreneurs presented Stephanie with \$109, she told them that



Bella Griggs

every 50 cents is one step closer to a cure. The girls promise they'll be back next summer, selling lemonade to help cure FA. One cup = one step closer.

Ultrahike for FARF

Peter and Janice Pless and friend, John Sutton, aka Team Julia, took on a tall order to raise funds for FA research. Last October, they participated in the Ultrahike, an annual event covering 26 miles of rough terrain on Bald Eagle Mountain in Pennsylvania. The Pless' daughters—Victoria, 20, and Julia, FA, 15—wrote a Facebook post during the climb that says it all: "The weather at the Ultrahike was horrible. Rain all day and cold temperatures. But that didn't stop our hikers. Mom and John pushed through the first half. And Dad finished the entire thing! They wore their Team Julia shirts the entire time. So proud of them!"



John Sutton, Peter and Janice Pless

Team Julia trained hard to prepare for their challenge and mailed around 500 fundraising letters. As a result, the Ultrahike raised more than \$11,700! The Team's motto? "Helping the FAmily one rock at a time."



KATA Foundation

The Kendall And Taylor Atkinson Foundation (KATA), founded by Ken and Jeanne Atkinson in memory of their children, supports effective

treatments and a cure for Fanconi anemia through the Fanconi Anemia Research Fund. Along with selling jewelry and running marathons, the KATA Foundation raises a majority of its funds at its annual Bravehearts Hoot 'n' Holler event, organized by the Atkinsons and Jack and Lisa Nash. As a result of last fall's record-breaking fundraiser, the KATA Foundation donated \$173,000 to the Fund in January: \$88,000 for research, \$45,000 for the Fund's scientific meeting on squamous cell carcinoma in March, \$30,000 for the Family Meeting scholarship fund, and \$10,000 for the 2012 FA Scientific Symposium! Our deepest gratitude to the Atkinsons, Nashes, and the many involved in the KATA Foundation.

Shoot FA

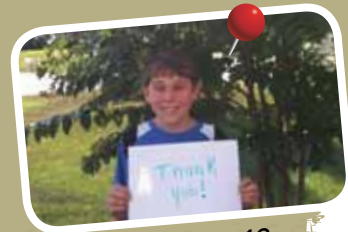
The DeHaan family organized its First Annual Shoot FA Benefit last September. Held at a sportmen's club and blessed with perfect weather, the fundraiser included skeet and trap shooting and clubhouse activities followed by a dinner and raffle. The DeHaans prepared invitations and flyers, set up a registration website, and secured sponsors and raffle items. Their tireless efforts paid off. The event raised more than \$6,400—well over their goal!



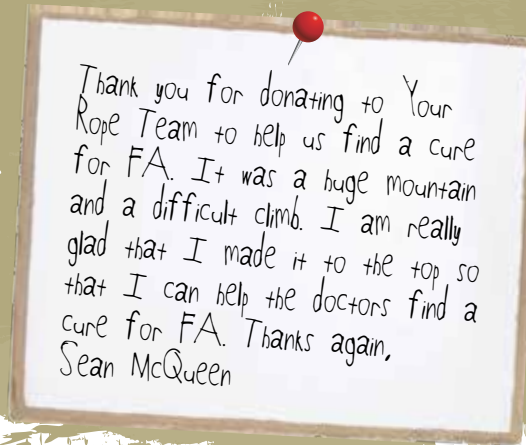
The DeHaan family



Your Rope Team, summit of Mt. Washington, June 28, 2011



Sean McQueen, 12



Dylan's Chuckwagon



Dylan Moore loved to cook and studied the Food Network channel. While he was undergoing a bone marrow transplant, his teacher assigned a homework project to come up with recipes, with the idea of publishing a cookbook for a few of the friends Dylan made while in the hospital. Although it took months, Dylan completed his cookbook. When all the doctors and nurses wanted copies of *Dylan's Chuckwagon*, Dylan and his family decided to sell the cookbook to help others with FA. Sadly, Dylan later died of transplant complications. The Moore family has sold 500 cookbooks to Dylan's fans across the globe. In addition to being a treasured family keepsake, *Dylan's Chuckwagon* raised \$1,500 towards a cure.

Family Fundraising Efforts Strong in 2011

In 2011, Fanconi anemia families, friends and supporters raised an amazing \$2,899,341. Every effort—letter appeals, silent auctions, races, themed events, yard and eBay sales—makes a difference. Funds were also donated in memory or in honor of someone special. Family fundraising also includes donations through the United Way, the Combined Federal Campaign, and an FA scientific community campaign. Thank you!

\$1,500,000 and up

Dave and Lynn Frohnmayr

\$250,000 - \$275,000

Gloria Miller

\$110,000 - \$149,999

Kendall And Taylor Atkinson Foundation
with the Nash and Atkinson Families
Glen Shearer and Peg Padden

\$40,000 - \$59,999

John and Kim Connelly
Kevin and Lorraine McQueen

\$30,000 - \$39,999

Steve and Jennifer Klimkiewicz/Wyatt's
Warriors

Robert and Barbara Capone
Peter and Tara Himmelreich
Fanconi Hope Charitable Trust

\$20,000 - \$29,999

Kerrie Brannock
Mike and Tracy Brannock
Ed and Janice Duffy
Kaps for Kendall
Matthew and Evelyn Keyes/Steven's
Association Moonrise
Todd and Kristin Levine

\$10,000 - \$19,999

Chris and Jennifer Branov
Chris and Susan Collins
Mark De Groot and Hanneke Takkenberg
Carole Felmy
Michael Glas
Brian Horrigan and Amy Levine
Charles and Katy Hull
Deane Marchbein and Stuart Cohen
Dan and Nikki McCarthy
Peter and Janice Pless
George and Kathryn Reardon
Kevin and Katie Rogers/My Best Friend

\$5,000 - \$9,999

Jimmy and Jenny Armentrout
Jeffrey and Donna Boggs
Donald and Danielle Burkin
Joseph and Nancy Chou
Darrel and Kalani DeHaan
Alan and Rachel Grossman
Roger and Eleanor Herman
Tanner and Jessica Lindsay
Jeremy and Stacey Mefford
Tyler Morrison and Rachel Altmann
Mark and Diane Pearl
Bob and Andrea Sacks

\$1,000 - \$4,999

Andrew and Vicki Athens
Mark and Linda Baumiller
Israel and Mary Jo Becerra
Randy and Nancy Bloxom

Richard and Tena Bosen

Patti Carter

David and Kim Chew

Donna DellaRatta

Brian and Jennifer Dorman

Lisa Doyle

Ezat and Laila Faizyar

David and Mary Ann Fiaschetti

Skip Longstaff and Susan Gannon-
Longstaff

Ben and Stephanie Griggs

David Guidara

Owen Hall and Margaret Kasting

Jeff and Beth Janock

John and Karilyn Kelson

Erik Kjos-Hanssen and Turid Frislid

Sejin Kwon and Jee-Ai Kim

Christopher and Dana Lamb

Mark and Angela Lamm

Gregory and Lynnette Lowrimore

Tue Marker and Kirstine la Cour

Rasmussen

Orion and Lisa Marx

Sheila Meehan

Gianna and Lauren Megna

Jim and Holly Mirenda

Sydney and Betsy Moore

Jack and Lisa Nash

Robert and Mary Nori

Ron and Fredi Norris

Joshua and Crystal Pepper

John and Dianne Ploetz

Paul and Rena Rice

Mark Ritchie and Lisa Mingo

Rick and Lynn Sablosky

Ron and Elesha Schaefer

Bill and Connie Schenore

Matt and Diane Senatore

Bryan and Karen Siebenthal

Mitzi Speelman

Bruce and Loreen Timperley

William and Mary Underriner

Mike and Beth Vangel

Marc Weiner

Sandy Weiner

Michael and Kim Williams

Up to \$999

Peter and Donna Abramov

Randy and Lauren Armstrong

Keith and Lily Baggett

Julie Barbier

Gerald Barbier

John and Audrey Barrow

Conrad and Joan Bender

James and Tracy Biby

Michael and Diane Bradley

Preziosa Briga

Lezlie Chesler

John and Launa Clanton

Tom and Mary Eileen Cleary

Tyler and Teresa Clifton

Natalie Curry

Brian and Margaret Curtis

Dottie Day

Richard Day

Jeremy and Michelle DellaValle

Wendy Delzell

John Delzell

James and Carol Dillon

Pat and Mary DiMarino

Antonino and Marie DiMercurio

Lindsay and Sandra Dunn

David and Kelly Dunnock

Gene and Lynn Eddy

Ginger Eggers

Sharon Ellis

Billy Jo and Debbie Estep

Kay Eubanks

Curt and Crystal Fales

Edwin Ferreira

Justin and Britteny Ferrin

Nancy Finnegan

Scott Finnegan

Jimbo and Candi Fisher

Doreen Flynn

Brett and Nanette Foster

Liz Funk

Gary and Melody Ganz

Brian and Lisa Gillott

Pat and Maria Gleason

Maria and Josh Godwin

Allen Goldbeg and Laurie Strongin

Andrew and Jennifer Gough

Young Ju and Kwang Sick Ha

Abdul Hameed

Racquel Hanna-Purser

Bob and Victoria Hathcock

Jeff Hoffman

Judy Hoffman

Shane and Colleen Irvin

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Lila Keleher

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Kayla Lackey

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Gail Richardson

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Severt and Beatrice Score

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Dan and Ashley Spradling

Tamara Stephens

Vicki Stephens

Greg and Brandi Stuart

Charles and Jennifer Sumrall

Paul and Debra Sundsvold

Mark and Susan Trager

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Van Es Family

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Norman and Michelle Wilson

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Jian Yang and Jing Nie

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For the fundraising events
calendar and current
FARF-funded research, visit
www.fanconi.org

Board of Directors Comings and Goings



Christopher Byrd; David Fiaschetti

The Fanconi Anemia Research Fund welcomed two new board members in February: Christopher T. Byrd, Esq., and David Fiaschetti, DDS. Christopher, an environmental lawyer for the Department of Environmental Quality in Tallahassee, Fla., is the first board member with Fanconi anemia. Christopher also represents the orphan disease community for the

United States Food and Drug Administration's Patient Representative Program. David is an oral surgeon in private practice in Portsmouth, R.I., and the parent of Peter Fiaschetti, a 12-year-old with Fanconi anemia. Christopher and David began a three-year term on Feb. 1.

We said goodbye to three board members in the last few months, each of whom gave many years of service to the Fund. Deane Marchbein, MD, joined the board in 1993 and chaired its Proposal Review Committee for 14 years before leaving at the end of January to focus on her leadership role in another important organization, Doctors Without Borders. Mary Ellen Eiler, a recipient of the Fund's Distinguished Service Award in 2008, resigned her board position in September 2011 after nearly 20 years of service both as a board member and executive director. Mike Vangel served faithfully for a dozen years; his board term also ended on Jan. 31. A heartfelt thanks to Deane, Mary Ellen and Mike for their passion for and commitment to the Fund's mission. ■

How FARF Can Help You Fundraise

More than 80% of the Fanconi Anemia Research Fund's annual budget comes from family fundraisers. The Fund's staff is here to help make your fundraising efforts a success. We can:

- Provide sample fundraising letters and help you edit and/or design your letter.
- Provide ideas, information and materials for events.
- Personalize your materials with your photo(s).
- Send your letter or invitation from our office using your mailing list.
- List your event on our website.
- Send a thank you letter and tax receipt to your donors.

All fundraising events should be covered by liability insurance. Insurance for a one-time event is often available through a homeowner's policy as a relatively inexpensive rider. Please contact the Fund if you need assistance obtaining or paying for this required insurance.

Please ask donors to write checks to the "Fanconi Anemia Research Fund." When we receive a donation, we mail a letter of thanks with a tax receipt, and we notify you that a donation has been made in your name.

We appreciate your fundraising efforts. You are making a difference. ■

New Funding Model Will Accelerate Research

The Board of Directors of the Fanconi Anemia Research Fund, along with its Scientific Advisory Board, made a significant decision at an annual planning meeting in January related to how the Fund will allocate resources for research in the future. The boards decided to explore a new model of funding that will accelerate Fanconi anemia research and that can more directly and rapidly impact the lives of people with FA.

Typically, the Fund receives unsolicited grant applications from researchers working in a university-affiliated lab. Now, the Fund will proactively seek applications for projects that reflect the strong need for collaboration and open sharing of information.

Considerable weight will be given to more immediately translational applications in the review process.

To reflect this new model, the combined boards revised the Fund's research priorities to include a statement of strategy and operating principles.

This document (see below) is a part of the grant application packet and is a factor used to determine whether a grant should be funded. We will be writing more about these changes as this new philosophy is integrated into the Fund's operations. By moving toward a more collaborative model with and among FA researchers, we hope to move closer to realizing our mission of finding more effective treatments and a cure. ■



Fanconi Anemia
RESEARCH FUND, INC.

Research Strategy, Priorities and Operating Principles

The Fanconi Anemia Research Fund seeks to improve the lives of individuals with Fanconi anemia through research that focuses on the rapid discovery and development of therapies or strategies that treat, control or cure Fanconi anemia. We have particular interest in funding highly collaborative, interdisciplinary, translational research efforts that effectively address one or more of the following priorities:

- To identify Fanconi anemia genes and their products, and to understand how inactivation of those products leads to the clinical manifestations of Fanconi anemia.
- To define the molecular pathogenesis of solid tumors in Fanconi anemia patients, and to develop strategies to prevent, treat and cure them.
- To determine the causes of bone marrow failure, myelodysplasia and leukemia in Fanconi anemia patients, and to develop strategies to prevent, treat and cure these disorders.
- To support the creation of shared resources and technologies for the international Fanconi anemia research community.

Operating Principles:

- We invite proposals from collaborative research groups and individual investigators that are responsive to the Fund's research strategy and advance one or more of the above priorities.
- We sponsor conferences and symposia with the specific goals of advancing knowledge, publicizing discoveries and fostering the broadest possible collaborations to advance Fanconi anemia research.
- We require all grant recipients and conference or symposia attendees to make data, models and tools rapidly available in the public domain to foster FA research.

Approved by Board of Directors 3/5/12

The Fund's 2011 Income and Expenses

Donations to the Fanconi Anemia Research Fund in 2011 totaled an impressive \$2,899,341. The \$1 million donation from the Richard & Mary Solari Charitable Trust (see article on page 21) and the \$253,000 gift from Mrs. Gloria J. Miller received last spring contributed to the near-record year, as did dozens of other efforts. Two examples among many are the \$971 we received from Benjamin Morrison's chess tournament and \$4,535 from the Lindsay family's Climb for a Cure. Thanks to all of you who held events, asked your friends and family or gave personal gifts. Every dollar you contributed makes a difference!

On the expense side, we spent \$917,458 on research grants and scientific meetings and \$173,858 on family support activities such as newsletters and the adult and family meetings.

The Fund's 2011 administrative costs, which include what we spent running the office and excludes the direct costs associated with family fundraising events,* were \$194,938, or 13.3% of total expenses. This percentage is a little higher than in previous years. It's important to note that the Fund's administrative *costs* have remained relatively constant for years. The *percentage* moves up or down depending largely on the number and cost of the research grants we fund within a 12-month period.

**Examples include paying for a tent or band for a family's auction or for postage for a fundraising letter. So that these costs are not a burden on families making an effort to raise funds, the Fund pays those expenses and must account for them in our books as expenses. The family fundraising expenses this year were \$49,880. ■*

Your FA Research Dollars at Work, 2010-2011

In addition to hosting a scientific symposium each year, and focused meetings on gene therapy (2010 and 2011), clonal aberrations (2010), and squamous cell carcinoma (2010), the Fanconi Anemia Research Fund awarded \$935,569 in research grants to the following projects in 2010 and 2011:

Investigator: Feng Chun Yang, MD, PhD, Indiana University Medical School, Indianapolis, Ind.
Title: *Investigating the Role of Microenvironment in the Development of Bone Marrow Failure in Fanconi Anemia*
Amount: \$81,950

Investigator: Farid Boulad, MD, Memorial Sloan-Kettering Cancer Center, New York
Title: *Multi-center Pilot Trial of HSCT Lacking a Genotype Identical Donor*
Amount: \$29,328

Investigator: Elizabeth Eklund, MD, Northwestern University, Chicago
Title: *Impaired Emergency Granulopoiesis in FA Leads to Immuno-deficiency and Predisposes to Acute Myeloid Leukemia*
Amount: \$255,000

Investigator: Julian Sale, MD, PhD, Cambridge University, Cambridge, UK
Title: *G Quadruplex-induced Epigenetic Instability in FA*
Amount: \$111,331

Investigators: Li Zhong, PhD, University of Florida College of Medicine, Gainesville, Fla.
Title: *Recombinant Adeno-associated Vectors for in vivo Gene Therapy of Fanconi Anemia (year two)*
Amount: \$100,000

Investigators: Josephine Dorsman, PhD, Free University, Amsterdam, The Netherlands
Title: *Identification of Pathway(s) Compensating for FA Gene Defects*
Amount: \$117,500

Investigator: Erich Sturgis, MD, MPH, The University of Texas MD Anderson Cancer Center, Houston
Title: *Prevalence of FA Gene Germline Mutations among Young Adults with Head and Neck Cancers*
Amount: \$240,460 ■

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: You can 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
Please go to www.fanconi.org to learn about other ways to donate.

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