



# FA FAMILY NEWSLETTER

#31

A Semi-annual Publication of the Fanconi Anemia Research Fund, Inc.

Spring 2002

## Family Meeting 2002

This year's Family Meeting will be held Friday, August 9, until Tuesday morning, August 13, at Camp Sunshine in Casco, Maine. The camp is located on beautiful Lake Sebago and is a 45-minute drive from the airport in Portland, Maine.

The Family Meeting provides the opportunity for families to attend scientific presentations by physicians and researchers discussing current, important issues in the research and treatment of Fanconi anemia. Topics

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## Fanconi Anemia Scientific Symposium

One hundred thirty-five researchers and treating physicians and ten FA parents met in Portland, Oregon, for the Thirteenth Annual International FA Scientific Symposium, November 14–17, 2001. Countries represented were Canada, France, The Netherlands, England, Spain, Germany, Italy, Israel, Brazil, Japan, and the United States. Forty-three researchers and treating physicians made formal presentations. Evaluations from the participants rated this as an outstanding symposium.

Research topics covered six areas: Diagnosis and Epidemiology; Animal Models; FA Protein Function and Hematopoiesis; FA Protein Complexes and DNA Repair; Bone Marrow Transplantation; and Gene Therapy.

Highlights included a number of presentations on the function of the FA proteins and how they are regulated and interact with one another. Scientists also presented their research on gene therapy for FA and on the results of their efforts to reduce the toxicity for FA patients undergoing bone marrow transplant.

Included as an insert in this newsletter is an outline of the Symposium presentations, giving the name of the presenter and the title of his or her presentation. If you would like a copy of one or more of these abstracts, please indicate your request on the enclosed form and return it to the FA Research Fund office. ♦

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## Auerbach Presents 20 Year Update

At the November Scientific Meeting, Arleen Auerbach of The Rockefeller University, New York, gave an update on data collected by The International Fanconi Anemia Registry (IFAR) over the past 20 years. A total of 802 FA patients are in the registry; 381 of these patients have been assigned to specific complementation groups.

Sixty percent are in FA-A, 23% in FA-C, and 13% in FA-G. These three groups account for 96% of all IFAR patients typed so far. Only a small number of patients have been assigned to FA-D2, FA-E and FA-F. Seventeen patients do not belong to any of the known complementation groups.

Auerbach analyzed 754 patients for cancer incidence (leukemia and solid tumors) over a 20-year period. This study revealed that 173 patients



(25.8%) developed the first malignancy at a median age of 14.5 (range, birth to 48.5 years). These 173 patients developed a total of 193 malignancies; 12 developed more than one tumor type. One hundred twenty (62%) of the malignancies were hematologic and 73 (39%) were non-hematologic tumors. Squamous cell carcinoma was the most common non-hematologic malignancy identified. The probability of developing a malignancy increases with age and, by 40 years of age, it approaches

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## Hadassah Hospital, Israel, Eliminates Irradiation in Transplants

Reuven Or, MD, reported on the results of seven FA transplants performed at Hadassah Hospital, Israel, since 1996. Patients ranged in age from three to thirty-one. Five had matched related donors (3 siblings, 1 mother, 1 cousin), and two had a matched unrelated donor (MUD). The preparative regimen included fludarabine, low-dose Cytoxan and anti-lymphocyte globulin (ATG). A higher dose of Cytoxan was administered to one patient in leukemic transformation.

All five patients with matched

related donors survived, including the patient with leukemia. One patient transplanted from a MUD died of infection. The second patient with an unrelated donor rejected the first graft. A second successful transplant from another unrelated donor was performed, using fludarabine, busulfan and Campath-1H.

Or concluded that fludarabine-based regimens are most effective in the conditioning of FA patients. This center believes that there is no need for the use of radiation. ♦

## Adult Stem Cells Hold Promise

FA patients, including those who survive bone marrow transplantation, are at high risk for developing solid tumor malignancies. Catherine Verfaillie, MD, director of the Stem Cell Institute at the University of Minnesota, believes that multipotent adult stem cells (MASC) may correct cells other than bone marrow cells in FA patients.

Verfaillie has been studying this special type of cell since 1997. These cells can now be isolated from the bone marrow. They are special because they can be grown in large quantities, can differentiate into a variety of different cells and tissues (e.g., liver, gut, lung), and will engraft in hematopoietic and epithelial tissues. When mice are given these cells, the MASC cells later appear in every organ, although in small quantities. When mice are irradiated prior to infusion of adult stem cells, the endothelial cells of their GI tract show an 8% engraftment of MASC cells.

Researchers at the University of Minnesota hypothesize that one could use an FA patient's own gene-corrected adult stem cells, or cells from a matching donor, to correct bone marrow and other tissues in the body. The University of Minnesota intends to pursue human trials of these cells initially in patients with Hurler's syndrome and FA. ♦

## FA and Cancer Conference

The FA Research Fund is sponsoring a conference to encourage research into the issue of the solid tumors—primarily head and neck, gynecological, and gastrointestinal—that affect FA patients. Through the leadership of Grover Bagby, MD, the chair of the FARF Scientific Advisory Board, and Blanche Alter, MD, MPH, from the National Cancer Institute, the Fund is convening a one-day meeting of leading cancer experts in Chicago on April 27, 2002. The focus of the meeting will be to define the issues and to determine the most fruitful and expedient course for the Fund to follow to advance the science relating to these

solid tumors. Eliane Gluckman, MD, from Hôpital St. Louis, Paris and John Wagner, MD, from the University of Minnesota, will attend to discuss post-transplant complications of FA patients. Researchers and clinicians from a number of cancer centers, including Johns Hopkins; Harvard School of Public Health; Dana-Farber Cancer Institute; MD Anderson; University of Chicago; Free University, Amsterdam; National Cancer Institute; Onyx Pharmaceuticals; and the Fred Hutchinson Cancer Research Center will attend this meeting to share their expertise with the Fund regarding this critically important issue. ♦

## Prenatal Diagnosis for FA

At the November Scientific Meeting, Sat Dev Batish, PhD, of The Rockefeller University, New York, gave a presentation on prenatal diagnosis for FA by families participating in the International Fanconi Anemia Registry (IFAR). Between 1985 and 2001, The Rockefeller University tested 80 pregnancies conceived with the hope of having an unaffected HLA-identical sibling. Fifteen percent of the fetuses tested were both unaffected and HLA-matched. A few families conceived multiple pregnancies before having a suitable sibling donor, while most failed to achieve this result.

Preimplantation genetic diagnosis with *in vitro* fertilization (PGD/IVF), described in previous newsletters, is a new option for these families. The Rockefeller University is aware of ten IFAR families (four in FA-C; six in FA-A) who have attempted PGD/IVF for conception of an HLA-matched, unaffected fetus,

resulting in three pregnancies. One of these attempts resulted in a cord blood transplant. A second mother has given birth to a healthy child, and a third mother recently achieved pregnancy. Some families have attempted this numerous times.

*Editor's note: FARF is aware of two PGD resources: Reproductive Genetics Institute in Chicago, and Wayne State University, Detroit. ♦*

## FA Handbook Available in Spanish

The third edition of *Fanconi Anemia: A Handbook for Families and Their Physicians* has been translated into Spanish. The book is available through the FA Research Fund office and may also be viewed on the FARF website, [www.fanconi.org](http://www.fanconi.org). ♦



Wolfram Ebell, MD

## Eliminating Radiation: Transplant Results in Germany

Wolfram Ebell, MD, of Humboldt University, Berlin, reported on 18 FA transplants at our recent Scientific Symposium. By eliminating radiation and Cytosan, Ebell hopes to reduce secondary tumors and toxicity. Engraftment is achieved by using fludarabine, high stem cell doses, T-cell antibodies, and low-dose busulfan. Ebell believes busulfan is effective in eradicating host cells and, therefore, abnormal clones.

Six patients received stem cells from matched sibling donors; twelve had alternate donors. Nine patients had severe aplastic anemia, six patients had myelodysplastic syndrome, and three had leukemia at the time of transplant.

All six patients with matched sibling donors, and eight of twelve with alternate donors survive. Of the four who died, three died of infection and one of leukemia. Toxicity was very limited. Five patients suffered graft failure after the first transplant. Four of these patients engrafted after the second transplant, and one after the third. Ebell has modified his protocol in an effort to prevent graft failures. Mean follow up post-transplant is 20 months. ♦

## Acute GVHD in FA Patients Transplanted from HLA Identical Sibling Donors – The Paris Experience

Philippe Guardiola, MD, Hôpital Saint Louis, Paris, discussed the incidence and severity of acute graft-versus-host disease (AGVHD) in 37 patients transplanted with bone marrow from HLA identical siblings. Transplants were performed in Paris between 1980 and 2000. Guardiola concluded that AGVHD is the leading cause of severe illness during the first months following transplantation. However, in spite of the high number of patients developing this

complication, only three died of AGVHD during the first 100 days post-transplant, all with grade III-IV AGVHD.

Fourteen percent of the patients did not develop AGVHD. However, 62% developed grade II-IV AGVHD, and 32% developed grade III-IV AGVHD. Approximately 43% of all patients were resistant to corticosteroids (resistance defined as unable to control AGVHD with 2 mg/kg/day) and required more

aggressive therapy (corticosteroid dosage of 5 or 10 mg/kg/day or an anti T-cell serotherapy) to control AGVHD.

In an effort to reduce the incidence of AGVHD, and because of the risk of secondary malignancies post-transplant, Paris is no longer using irradiation when transplanting patients with matched sibling donors. ♦

## John Wagner Reports on Outcomes and Risk Factors for Unrelated Donor Transplants

Between April 1999 and November 2001, 27 patients underwent an unrelated donor transplant for FA at the University of Minnesota Hospital. Of 24 evaluable patients, all engrafted. No patient developed grade III-IV acute graft-versus-host disease (GVHD); three patients developed chronic GVHD. Survival

depended upon the stage of disease. Patients with advanced myelodysplastic syndrome (MDS) or leukemia, history of systemic fungal infection, or gram-negative sepsis had a 13% survival. Sixteen of 18 patients without these risk factors survived at two years, a survival rate of 86%. Most of the patients transplanted in Minnesota had been on androgens at some point prior to transplant.

A retrospective analysis of 84 patients transplanted with unrelated donor bone marrow, facilitated by the National Marrow Donor Program, was performed to determine if there were risk factors that could predict engraftment and survival in FA patients. Factors reducing risk were younger age (per decade), pretransplant condition, absence of androgen therapy, and use of fludarabine in the pretransplant conditioning regimen.

Wagner concluded that fludarabine promotes engraftment and that transplantation prior to the development of advanced MDS, major infection or use of androgens may have a significant impact upon survival.



Certain patients, such as those over the age of 20, are at greater risk and should consider androgens prior to transplantation.

Based on these results, young patients (<20 years) with good organ function and an HLA matched bone marrow donor should consider treatment with marrow transplantation rather than androgen therapy (this is also recommended for those with an HLA matched sibling donor).

Patients who are older (>20 years), have an HLA mismatched marrow donor, or poor organ function, should consider a trial of androgen therapy since these patients have a higher rate of transplant complications. ♦

## Clinical Trials

The FA Research Fund has started a program of investigating clinical trials to locate those that may be of benefit to FA patients. Nicole Westrich of our staff will develop a Clinical Trials section for the [www.fanconi.org](http://www.fanconi.org) website, and she will post current clinical trial information in subsequent newsletters. If you learn of a trial that you feel may be of benefit to FA patients, please contact Nicole at [nicole@fanconi.org](mailto:nicole@fanconi.org) so that she may investigate the trial and publish any promising results for the benefit of all. ♦

## Gene Therapy Trial Planned

Frank Smith, MD, Children's Hospital Medical Center, Cincinnati, described an international, multi-center pilot study to determine the safety, feasibility and efficacy of gene therapy for the treatment of FA. The study will use a retroviral vector to transduce hematopoietic stem cells obtained from the patient's bone marrow, cord blood or mobilized peripheral blood progenitor cells. This three-year trial plans to enroll 10 FA-A patients, and five FA-C or FA-G patients.

Patients eligible for this trial must be age 6 or older, in complementation groups A, C or G, and must have normal bone marrow cytogenetics (absence of an abnormal clone) for the previous 12 months. Patients with myelodysplastic syndrome (MDS) or acute myelogenous leukemia (AML) will not be allowed in the study. Participating centers include Children's Hospital Medical Center (Cincinnati), Fred Hutchinson Cancer Research Center (Seattle), Riley Children's Hospital (Indianapolis), St. Jude Children's Research Hospital (Memphis) and Düsseldorf Children's Hospital (Germany). Smith anticipates that this trial will be open to patient enrollment during the next year. ♦

## Website Offers Comprehensive Review of FA

Akiko Shimamura, MD, PhD; Lisa Moreau, MS; and Alan D'Andrea, MD, of the Dana Farber Cancer Institute have posted a comprehensive, up-to-date review on Fanconi anemia on the GeneClinics website. This lucid resource should prove helpful to anyone searching for an overview of our current knowledge concerning disease



## Evidence for Additional Complementation Groups

Hans Joenje, PhD, Free University Medical Center, The Netherlands, now suspects that there are more than eight FA complementation groups. His lab has identified 8 cell lines that do not appear to belong to any of the previously described groups. Joenje's best estimate is that these cell lines may fall into 2 to 4 new groups, which would imply a total of 10 to 12 FA complementation groups. An estimated 1 out of 25 patients may belong to one of these new groups. Cloning the genes represented by these new groups is important, since these genes could shed light on the function of the FA proteins. Joenje's lab is trying to find more patients belonging to these new complementation groups, which will facilitate identification of the corresponding genes. ♦

## Harris Reports on 10 Alternate Donor Transplants

Rick Harris, MD, Children's Hospital Medical Center, Cincinnati, reported on 10 FA patients who underwent transplantation with an alternate (not a matched sibling) donor at his center. The preparative regimen consisted of low-dose Cytoxan, total body irradiation, anti-thymocyte globulin (ATG) and fludarabine. Donor cells were T-cell depleted.

Five patients received bone marrow; five received peripheral blood stem cells. Only those patients

receiving bone marrow were given cyclosporine post-transplant. Three patients had a 5/6 match; seven a 6/6 match (two of the 5/6 matched patients were also mismatched at the C locus). Five patients had previously undergone androgen therapy.

Eight of these ten patients are alive from one to 29 months post-transplant. All five patients receiving peripheral blood stem cells are alive, and none of these five experienced graft-versus-host disease (GVHD). Of the five patients receiving bone marrow, three survive. Three of these patients experienced significant GVHD. One patient died of grade 3 GVHD and aspergillus fungal pneumonia; another died of early pulmonary failure. This protocol is being modified to eliminate the T-cell add back in bone marrow recipients in an effort to reduce the risk of GVHD. ♦

characteristics, diagnosis, complementation groups, and therapies for FA.

To access this website, go to <http://www.geneclinics.org>. First time users need to follow a simple registration procedure. After you register, go to GeneClinics and type Fanconi Anemia by the Search box. You're there! ♦

# The Androgen Issue: Some Reflections

by N.T. Shahidi, M.D., Emeritus Professor, University of Wisconsin Pediatric Hematology/Oncology

*Many of you remember Dr. Nasrollah Shahidi, who spoke at our family and scientific meetings prior to his retirement and has guided many physicians, parents and FA patients in devising treatment plans. We asked him to comment on the issue of androgen therapy for patients who lack a matched sibling donor. His response is below:*

At a meeting organized by FARF in May 1998, participants agreed that all newly diagnosed FA patients without an HLA-matched sibling donor should be started on oxymetholone upon onset of bone marrow failure. Periodic review of standard care and necessary changes by consensus are essential for better outcomes in any disorder. Recent preliminary reports indicate that conditioning regimens which include fludarabine may result in a significant improvement in the outcome of FA patients receiving unrelated hematopoietic cell transplantation. Since the major benefit was seen primarily in those who did not have advanced myelodysplasia or prior infection (standard risk patients), it has been advocated that, instead of androgens, unrelated matched transplantations should be performed as standard therapy for some FA patients.

While the results are encouraging, two-year survival data in a small number of patients does not provide a solid foundation for changing the 1998 consensus recommendation for standard care for patients without a matched sibling donor.

It should be pointed out that ablation therapy with radiation and other carcinogenic agents in conjunction with highly immunosuppressive agents such as fludarabine and ATG in patients receiving T-cell depleted bone marrow would obviously result in a profound and long-term immunodeficiency. Aggressive antiviral and antifungal therapy may protect the patients from infection, but cannot prevent carcinogenic effects

of radiation and Cytoxan on highly cancer-prone FA cells. Adequate cell-mediated immunity is an important factor in prevention of proliferation of mutated cells.

A study of 700 patients (1,2) with severe aplastic anemia, including Fanconi anemia, transplanted in Paris or Seattle showed that the most common post-transplant cancer was squamous cell carcinoma. Statistical analysis revealed that two important factors were diagnosis of Fanconi anemia and therapy with azothioprine as an immunosuppressive agent given to control GVHD. The squamous cell carcinomas occurred 2-1/2 to 18 years (median of 8 years) after transplantation. Other less serious post-transplant complications in FA patients are endocrine disorders and esophagitis, esophageal stenosis, and GVHD.

The current standard of care advocating the use of androgens is based on the fact that the vast majority of FA patients (approximately 75%) respond to androgens. The response to androgens is not an "all or nothing" phenomenon. Some patients show a rapid and sometimes dramatic response within a short period and may experience abnormally high hemoglobin if the androgen dosage is not reduced. Others may respond to higher dosages and only after several months.

Some of the androgen-responsive patients can be maintained on reduced dosages for decades without significant untoward effect, such as permanent liver pathology. Some have become androgen independent. Androgens have been used in the

therapy of FA for the past 45 years and have resulted in a significant improvement in the survival of these patients. Many preparations have been used in the past. Some of the testosterone derivatives, such as testosterone esters or fluorinated preparations such as Halotestin®, are highly virilizing and should not be used in children and women. Among the so-called anabolic steroids, oxymetholone has proved to be the most effective hemopoietic stimulant and remains the only FDA-approved androgen for the therapy of bone marrow failure syndromes. Danazol, an attenuated androgen with immunoregulatory properties, is commonly used in immune thrombocytopenias, lupus erythematosus, and endometriosis. Some of the androgen-responsive FA patients may respond to Danazol.

Oxymetholone, when judiciously used, does not cause any major side effects. Blood-filled empty spaces in the liver, known as peliosis, and hepacellular carcinoma are rare and occur only in patients who have been on oxymetholone over several years, in large doses (3). The presence of hepatitis C and B have been additional factors. Cholestatic jaundice and elevation of liver enzymes, if they occur, are reversible.

In view of the above, we should continue to use androgens as standard therapy for those who do not have an HLA-identical sibling. This could allow the androgen responders many years of hematologic remission and will give the parents time to select other options such as

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## The Androgen Issue: Some Reflections

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pre-implantation genetic diagnosis, gene therapy, or less toxic approaches to bone marrow transplantation.

We also have to make sure, by developing a detailed guideline for androgen therapy, to avoid hepatic lesions and to identify the non and/or poor responders before the onset of severe pancytopenia, advanced myelodysplasia or AML, so they are better candidates for unrelated matched hemopoietic cell transplantation.

Finally, I would like to urge the interested members of major pediatric transplantation centers to create a study group and use mutually agreed upon randomized protocols for hemopoietic cell transplantation in FA patients. Such an approach would result in a larger number of similarly treated patients within shorter periods of time and answer important questions such as whether fludarabine could obviate the need for radiation. We are dealing with an orphan disorder, and competing for a small number of patients is good neither for the families nor for medical progress.

### Selected Readings:

“Malignancies after marrow transplantation for aplastic anemia and Fanconi anemia: a joint Seattle and Paris analysis of results in 700 patients.” *Blood* 87:386-392, 1996.

“Malignancies after hemopoietic stem cell transplantation. Many questions. Some answers.” *Blood* 91:1833-1844, 1998.

“A review of the chemistry, biological action and clinical applications of anabolic androgenic steroids.” *Clinical Therapeutics* 23:1355-1390, 2001. ♦

## Grover Bagby Receives Distinguished Service Award

At the Presenters' Dinner at the Thirteenth Annual Scientific Symposium, Grover Bagby, Jr, MD, of the Oregon Cancer Institute and the chair of the FARF Scientific Advisory Board since its inception, received the Fund's first Distinguished Service Award. Dave Frohnmayer, who presented the award to Bagby, noted that the award was given with gratitude and profound appreciation for unparalleled scientific and organizational leadership on behalf of the Fanconi Anemia Research Fund and Fanconi anemia patients worldwide. In voting to make this award to Bagby, members of the Board of



Directors of FARF noted his remarkable professionalism and the selflessness with which he provides endless support to the Fund. Board members also noted that, despite the fact that Bagby is very much involved in research, he clearly keeps the visage of FA patients in the forefront as he provides leadership in the FA research community. Congratulations and thank you, Dr. Bagby! ♦

## Patients with Radial Ray Deformities Need Follow-Up Care

*By Blanche Alter, MD, MPH*

Adults who have radial ray deformities may develop painful consequences (bone and joint problems, arthritis, etc.) from years of using their hands differently than the rest of us. Many of us have been unaware of this problem.

I recently saw an adult with FA who has had years of wrist pain. Any of our patients with hand or thumb problems, whether or not they have had surgery, ought to see orthopedists, rehabilitation medicine, or physiatrists on an ongoing basis, and also get early and continuous advice from physical and occupational therapists. Doing surgery, or not doing surgery, should not be the end of the relationship. ♦

### Auerbach Presents 20 Year Update

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54% for hematologic malignancy and 52% for solid tumors.

Auerbach stated that there are significant differences between patients in FA-C and patients in FA-A and FA-G. In FA-C, the age of onset of hematological problems is younger, and overall survival is generally poorer. However, there was no significant difference in overall survival time between groups A and G.

FA-C patients were analyzed according to their specific mutation. They were divided into three subtypes: (1) patients with IVS4; (2) patients with at least one exon 14 mutation; and (3) patients with at least one exon 1 mutation and no known exon 14 mutation. The overall survival for patients in groups 1 and 2 compared to group 3 was significantly worse. ♦

# NCI Study Launched on Rare Inherited Bone Marrow Failure Disorders and Cancer Risk

The National Cancer Institute (NCI) is launching the largest North American study of its kind to focus on people with rare inherited bone marrow failure syndromes (IBMFS) and their immediate family members. This study, called the “NCI IBMFS Cohort,” will follow families over a long period of time, and examine the underlying genetic disorders of those diagnosed with IBMFS and their families, and analyze how certain factors can affect the course of these syndromes. Families with these disorders are invited to become part of the study, since they may be at a higher risk of cancer. These families can include affected individuals and their immediate family members, as well as surviving relatives (in families where the patient may have passed away) who may be carriers of one of the altered genes related to these diseases.

IBMFS, most often diagnosed during childhood, are relatively rare disorders that involve some form of aplastic anemia. People with these syndromes are at increased risk of cancer such as leukemia or various specific solid tumors. The study will enroll families in which at least one member has or had an IBMFS such as FA.

One problem for families living with these conditions is the impending threat of cancer in young patients, without knowing whether it will happen or when it will happen. The challenge for researchers is understanding why cancer develops in so many people with IBMFS, why it occurs earlier than in the general population, and what the role is of IBMFS genes in carcinogenesis.

“By looking at a large group of patients and family members who

may be cancer-prone, we hope to learn more about these issues, and to evaluate techniques for cancer screening and prevention in this particular group,” comments Blanche Alter, MD, MPH, the principal investigator at NCI. Alter has teamed with a large number of associate investigators in all specialties at the National Institutes of Health and at several medical centers in order to provide a truly comprehensive evaluation to persons with these complex, multi-system disorders.

The investigators hope to enroll all North American families with these syndromes. There will be two subgroups—those who are seen and evaluated at the NIH Clinical Center in Bethesda, Maryland (called the “Clinical Center Cohort”) and those who provide medical information but are not seen at the Clinical Center (called the “Field Cohort”).

Affected individuals and their immediate family members who come to the Clinical Center will receive comprehensive physical and laboratory examinations by a team of specialists, along with information and advice regarding the management of any newly identified clinical problems that are detected during the course of their visit. Due to the high risk of cancers in this cohort, participants will be offered age-appropriate, thorough cancer surveillance as part of the study. At the participants’ request, they will be given the results of clinical tests and cancer screening. The study will not provide treatment at the NIH; patients will be referred to their physicians for consideration of any treatments that may be necessary.

For further information about the study “Etiologic Investigation of Cancer Susceptibility in Inherited

Bone Marrow Failure Syndromes (IBMFS),” interested individuals may call 1-800-518-8474 to speak to Lisa Leathwood, the study’s research nurse, or send an email to [lisaleathwood@westat.com](mailto:lisaleathwood@westat.com). More information is also provided on the study website at <http://www.marrow-failure.cancer.gov>. ♦

## Transplant Results from Curitiba, Brazil

Ricardo Pasquini, MD, Hospital de Clinicas, Curitiba, Brazil, reported on a study of 45 FA patients transplanted at his center. Patients were in aplastic phase, without myelodysplastic syndrome (MDS) or leukemia. All had HLA compatible relatives (44 had matched sibling donors; one donor was a grandfather). Forty-two patients were given bone marrow; two received peripheral blood stem cells; one received cord blood. Radiation was eliminated from the protocol. Methotrexate and cyclosporine were given to prevent graft-versus-host disease (GVHD).

This study compared patient outcomes at decreasing doses of Cytoxan. Eight patients received 120 mg/kg of Cytoxan; 19 received 100 mg/kg; six were given 80 mg/kg and 12 received 60 mg/kg. Four of those receiving the highest dosage (50%) survive, compared to all twelve who received the lowest dose. The median post-transplant follow-up of the lowest dose group is 15 months, ranging from 4 to 32 months.

Pasquini concluded that lower doses of Cytoxan were associated with better survival, less toxicity, and lower incidence of both acute and chronic GVHD. ♦



# From Participants' Evaluations of the Scientific Symposium

*"Excellent. Superb meeting."*

*"Excellent. My 1st meeting. I was very impressed. It ran very smoothly and forum discussions were lively and interesting."*



*"Excellent. The way the basic science and clinical aspects of FA were put together was excellent and is a strong point of this meeting."*

*"Excellent. Presentations short and precise."*



*"Excellent. You have it about right."*

*"I think it is excellent overall. As it was my first FARF meeting, I really enjoyed the mixture of presentations!"*

*"Excellent format, which fosters interactions in informal atmosphere."*

*"Again excellent. I have enjoyed the meeting VERY much."*

*"The meeting included a good balance of researchers representing the different critical objectives of the FARF."*

*"Excellent. I like attending this meeting very much. It is well organized, very reasonable number of attendees where person-to-person interaction is possible."*



## My Children, My Angels

by Kelly Bennett

Our story started 4 years ago when we found out that our 4-year-old son, Marshall, who has no obvious anomalies, had Fanconi anemia. At that point we had decided not to have any more children. Isn't it funny how life works? Almost 2 years later, I became pregnant again. When I was 18 weeks along in my pregnancy, we found out that we were having a little girl, Amelia Angelica, and that she, too, had FA. The ultrasound showed that her arms were clubbed. At first, I was devastated. I'm still not sure if it was because she had FA or because she had a deformity. I know how the world is and how people can be cruel. But when Amelia arrived on June 6, 2000, my whole world and perspective changed! There before me was the most beautiful little girl I'd ever seen. The funny thing is I didn't notice her arms because, no matter what, she was perfect.

While in the hospital, we discovered that she had one well-functioning kidney and a few holes in her heart, which have since closed. She also had bi-radial clubbed hands and missing thumbs.

Today she is almost 2 and doesn't let a thing stop her. She just spent 3 months in a cast due to hip dysplasia that was diagnosed when she was 18-months old. When she gets her cast off, she will spend 18 months in braces. Amelia will also be having surgery soon on her hands to centralize both of them, which has been a tough decision for us. We hope that we are making the right decision. We will not be having both index fingers rotated, just the right one.

My children consume most of my life. I don't work or, at least, I don't get paid money for my work. Marshall is unable to attend school. We have a homebound teacher who



Marshall and Amelia Bennett

comes in 3 times a week. On Thursdays we travel 90 miles round trip for platelet transfusions. Amelia has therapy 3 times a week with an occupational therapist, physical therapist, and a speech therapist. People ask us daily how we do it. I really don't know how to reply. I am only doing my job. I love my children. I do it because this is what was chosen for me. They are my angels. I truly feel that I am the special one God has chosen to raise His angels and guide them the best that I can. ♦



Clay Williams, Matt, Andrea and Dalton Morris

## Attending the FA Family Meeting for the First Time

by Andrea and Matt Morris

Thanks to the scholarship program provided by FARE, we were able to attend our first family meeting last August in Lake Geneva, Wisconsin. The scholarship provided us with lodging, meals, and reimbursement for our gas roundtrip. Even though we were there on the scholarship, we were treated just like everyone else. We urge anyone who needs financial aid in order to attend a family meeting to contact FARE.

Our first Fanconi Anemia Family Meeting was a wonderful experience! We got to meet so many nice people, who were more than willing to listen to us, help us to understand the things that we have been going through and will be going through, and to share their experience with us. The kids affected with Fanconi were incredible to watch and to listen to. Their positive attitude and their

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## The Burkin Family's Experience at the FA Family Meeting

by Danielle Burkin

Anxious, nervous, excited. Just three emotions among many others my husband Donny and I felt prior to the 2001 Family Meeting. This was to be our first meeting—our daughter, Hope, had been diagnosed with FA in January of 2001.

Upon arrival at Lake Geneva, we immediately knew we made the right decision in attending the meeting. The speakers were fantastic. They helped clarify many of the things we had read about FA, and they were always willing to stick around to answer the numerous questions many of the families had after the presentations.

Just as beneficial to us was the way the families opened their arms to us to share their experiences, grief, joys and frustrations. Through the experiences of other families the journey of dealing with FA is made so much easier. You no longer feel alone in fighting this terrible disease.

Donny and I took away two very important things from the Family Meeting. The first was fundraising—how important it is to raise money to help find a cure for FA. Second, we had a clearer understanding of what we had to do to take care of our little girl. We realized just how much our doctors did not know or understand



Hope Burkin

about FA and how important it is to start asking better questions and pushing them for better answers.

We recommend this camp to anyone who is close to an FA patient, newly diagnosed or not. The feeling this “family” brings to you is truly special. We look forward to the camps to come and hope that many more families will be able to make it. ♦

## Molly Nash Update

by Lisa Nash

On September 26, 2000, Molly received a cord blood transplant from her newborn brother, Adam. We prayed that this would be the start of a new chapter in her life. We would have never guessed that her life would be this great 500 days later. She is HEALTHY for all intents and purposes.

Molly goes to school full-time without any restrictions. She belongs to Brownies and Student Council and is in soccer and dance, which she loves. She will never be an Olympic soccer star, but she is having fun anyway. She is learning to ride a horse, which has always been her dream. She is very good at this, and we've promised her that she can have her own horse when she is ten.

Molly has more energy than she has ever had in her life. She never gets tired and is now able to do everything that a 7-year-old does.

She is doing great in her first year of school, but doesn't like going to time out for trying some words out on Mommy that she learned in school! Molly picks up every cold and virus there is, but the great thing is that she gets better when she is sick. She still isn't eating though, is fed by a feeding tube and pump at night, and gets feedings at school. But she has come soooooo far! She is in a brace for severe scoliosis. We pray that the brace will do its job and no surgery will be needed. She is still having some liver issues from the transplant but, with each visit to the bone marrow doctor, it is getting better.

Molly loves and adores Adam most of the time, except when he is



Molly Nash with her painting

in her stuff (which is most of the time). She watches out for him like a mother hen. They are adorable together and have a bond stronger than most siblings. We can truly say that God has blessed us with two miracles. Not a night goes by that we don't thank God, the doctors and nurses, and all the people who prayed for Molly and Adam for allowing this

*continued on page 13*

# Long Term Coping With Losing A Child

by Mike & Pam McCoury, Burlington, North Carolina

Our precious son, Christopher Michael McCoury, was diagnosed with Fanconi anemia in June 1990 at the age of three. Four years later, in June 1994, our universe caved in on us when our courageous little 7-year-old warrior took his last breath and passed away. The chicken pox, which he got about five months earlier, was a contributing factor to his death. It so weakened his immune system that, over time, he was unable to fight off any infections that came his way.

Those last five months he lived we tried to lead as normal a life as possible. He would start getting sick more often and that almost always meant a stay in the hospital for a couple of days. When we got home from the hospital he would ask, "Dad, can we play ball?" Even at 7 years of age and battling this disease, he still wanted to TRY to do things he enjoyed, even though he knew he wouldn't have the endurance that he once had.

It will soon be eight years since we lost Christopher. So how do we cope with the long-term effects of all this? Both Pam and I have good days and bad days. Birthdays and holidays continue to be a struggle. Losing your child, especially a child at the age of seven, is not the natural order of things. You just expect your child to live longer than you. However, our faith in God and our continuing to focus on Him is what has sustained us through those down times. As we experience sadness because Christopher is not with us, it is turned to joy because we know that he is sitting on the lap of Jesus. As much as we would love for him to be here with us, we also realize that there is no better or safer place for him to be.

One of the many things Pam and I have learned through all this is that, when a non-life threatening crisis situation develops, we don't see it as a crisis but as a minor inconvenience to our daily lives. Whether it is a job loss or ruptured water pipes, these things are temporary and will pass. Additionally, we understand the significance of one sunrise and one sunset every day. Take things one day at a time and enjoy each day for what it is—a miracle and a blessing from God.

When people learn that we have lost a child, they almost always avoid those conversations involving children or will quickly change the subject for fear that it will hurt us or cause us undue pain. Quite honestly, just the opposite occurs. Nothing gives us more joy than the opportunity to talk about Christopher, just as any parent would want to talk about their child. Not giving us that chance would be denying that he ever existed. And, oh, how Christopher lived the seven years we had him. How he did live!!!!

Back in June 1990 when we learned from our doctors that little was known about this disease, we felt so alone and isolated. We had no idea how to go about getting information to learn what it was we were up against or trying to imagine what kind of obstacles Christopher would have to overcome later in life. We could barely even pronounce the name, let alone do research. Two weeks later at our next visit to the UNC Hospital in Chapel Hill, our doctor gave us a phone number for a group called the Fanconi Anemia Research Fund. He told us that these folks were a wealth of information and could answer our questions. We



*Christopher McCoury*

talked with a very precious lady named Lynn Frohnmayer and were amazed as to the knowledge that Lynn possessed about this disease. It was during that conversation that we got the wake-up call concerning the serious nature of Christopher's situation. Because of the efforts of the FARE, we were able to ask more intelligent questions of our doctors and staff. Without constant feedback from FARE and other FA families during those years of androgens and blood transfusion therapy, life would have been miserable. We knew that at any time we had people we could call just to talk concerning blood counts, platelets, etc., and that they would understand what we were talking about. We cannot express how grateful we are for the FARE, in particular Dave and Lynn Frohnmayer for their tireless and diligent efforts in making this organization possible and the annual family meetings that they organize. These are a Godsend!!

In closing, we would encourage all parents, whether their child is affected with FA or not, to cherish and love your child with all your heart. Spend time with them, love them, laugh with them, and cry with them when they hurt, because there is no guarantee they will be with you tomorrow. ♦

## A Tribute to Nikki

by Beth Losekamp

When I think of my daughter Nikki, I remember a beautiful young lady who loved life. Hardly ever did anyone see her without a smile on her face and a book in her hands. She had advice on everything. Of course it all made perfect sense. Wise beyond her years and just as talented, Nikki was a true hobbyist if ever there was one. I truly believe that her passion was in making jewelry. She was just starting her own line when she was taken away. She was planning on donating a portion of the monies made from her jewelry to FARE. Nikki and her friends were always planning fundraisers. She "didn't want her brother to go through all this hard stuff."

Nikki was never one to complain about anything. She always said, "It's better that this happen to me because I can handle it. Why should anyone else have to deal with it?" Nikki was/is everything to me. From the day she was born she gave me more

joy and inner strength than I ever thought possible. She stole everyone's heart. So many people have told me what a "true inspiration she is." She is gone now, just a day after her fourteenth birthday. Her little brother as well as her father and I are truly lost without her. Her inspiration will live on. Her zest for life now resides in us; we will have to live for Nikki. We will have to keep her smile alive for all to see.

Nikki didn't want to die so young. She had dreams of her own: growing up, having a large home with lots of animals, and living close to an ocean. When the aspergillus planted its nasty self in her brain we had to make a lot of unpleasant decisions. Nikki would have fought a very long time if it weren't for that. Once it started spreading, she went to sleep on a Friday night and did not wake



Nikki Losekamp

again. She died on that Sunday morning at 4:25. I miss her deeply, my best friend. Now God has my "Little Angel Baby" as his friend. Until we meet again Sis. We love you. ♦

### Molly Nash Update

*continued from 11*

miracle to happen. At the same time, we pray for all of the other families who have been touched by FA and pray that someday there will no longer be a disease known as FA. ♦

## In Loving Memory

Mallory Bowser  
5/12/73-5/14/01

Patience Lee Curry  
10/12/78-12/12/01

Morena de Leon  
10/19/77-1/4/02

Stephen Fitzgerald  
9/12/91-11/26/01

Nikki Losekamp  
12/15/87-12/16/01

Travis Massino  
1/1/81-2/4/02

Simon Rasmussen  
7/26/01-12/17/01

Nadine Scheloske  
5/11/82-12/01

Cole Smith  
9/12/99-11/27/01



## The Gift

by Donna J. Boggs

Children, like packages, come in many shapes and sizes. Each one is unique and created solely with the recipient in mind. Dad's eyes, mom's mouth and chin, dad's hair color, and mom's sense of humor. Each cell takes on specific characteristics that separate each child from the other, yet is fashioned in the master's own image.

The first time I laid eyes on my baby, Nicholas, he was wrapped in a blanket with a nursery cap on. All I could see was a beautiful, little round face. The next time I saw him was in the neonatal intensive care unit. He had undergone surgery to repair a tracheal-esophageal fistula. Tubes and wires were going in every direction. I saw a little black-haired, dark brown-eyed angel who had arms shorter than the other babies, hands turned in toward the arms with no thumbs, and four fingers on each hand. He was beautiful. I was ecstatic. The only place I could kiss was his little toes, which immediately clamped around my upper lip. With tears of

joy, I prayed and begged God to let him live.

This package is called Fanconi anemia. The contents of this package are different on the inside also. His esophagus wasn't connected to his stomach. Instead it had grown into his windpipe. His stomach is extra sensitive. He had reflux, aspirated easily, and needed a g-tube for feedings. He needed a tracheotomy so he could breathe easier. He had paralyzed vocal cords and only one kidney. He had hydrocephalus and had to have a ventricular shunt. He also has asthma. He is underweight and not as tall as others his age. He is in bone marrow stress which will go into failure and require a bone marrow transplant. He is at risk of cancer for the rest of his life.

They call these differences anomalies. I call them wrapping paper,



*Nicholas and Spencer Boggs*

ribbon, bows, and angel decorations. Each day is blessed. His will is of iron and his heart of gold. His laughter creates a sparkle in his eyes and in yours. He has a special effect on others and they never forget his sweet disposition. I will always treasure this precious gift created just for us. ♦

## An Angel Among Us

by Chris and Amanda Smith

To know Cole was to know love. He shared his love with everyone he met. I will never forget when Cole was first diagnosed with FA at the young age of 11 months. Chris and I were two young parents who were just getting to know our precious baby when our lives began to revolve around a disease called Fanconi anemia. Cole was immediately introduced to a world of doctors, nurses, hospital rooms, and—our least favorite—needles. He had blood draw after blood draw and test after test. To our amazement, he handled

all of this with a strength and bravery that few ever know. He would be crying and upset with the nurses one minute for sticking his arm and smiling at them the next, because that was just Cole. His smile could melt the hardest heart.

Cole touched many lives in his two short years here on earth. His presence is still felt by many who knew him. He taught those around him to never give up. We lost Cole in body, but not in spirit. His spirit will live on forever. He was an angel here on earth as he is now in Heaven.



To Our Precious Angel, Cole:  
We love you,  
Momma and Daddy ♦

# FA SUPPORT GROUPS

## An Update from Fanconi Canada

by Lorne Shelton

In December, Mary Heath and family near Toronto hosted our annual Holiday Happening. Several FA families were in attendance and enjoyed a wonderful evening together.

Last November, members of Fanconi Canada attended an exploratory meeting sponsored by the Anemia Institute for Research and Education regarding the formation of a “joint-working group” of individuals and organizations dedicated to the improvement of lives for those with anemia. The meeting brought together the various anemia communities, including thalassemia, sickle cell anemia, aplastic anemia, myelodysplasia, celiac and lupus. We explored a number of ways in which we could move forward to achieve common goals. One particularly exciting initiative has come out of the working group.

The Canadian Hematology Society’s annual conference will be held in April in Montreal. The CHS is devoting this coming year’s conference to “serious” chronic and congenital anemias, including a lecture on clinical management of FA.

Fanconi Canada has entered into an agreement with the Canadian Institutes for Health Research to fund a research fellowship on FA at the postdoctoral level. The jointly funded grant is for \$38,500 per year for three years.

We are off to a great year in fundraising thanks to FA families from coast to coast in Canada. We are putting the finishing touches on another fundraiser for May in Toronto called MAIN COURSE...MURDER featuring a murder mystery dinner theatre and silent auction. ♦

## FA in The Netherlands: The Dutch FA Support Group

by Ron Baas

In The Netherlands we have about 30 FA families, with patients ranging in age from one to 63. Several patients have had a successful bone marrow transplant, but there are also patients with a mild type of FA who don’t need a transplant or medication.

The Dutch FA Support Group was founded in 1993. Since 1998 there has also been a subgroup from the VOKK (Foundation for Parents, Children, and Cancer), which is a

large organization for the families of children with cancer. Once a year the Dutch FA Support Group comes together to meet each other and share experiences in dealing with FA and to hear speakers on FA research and treatment.

Every three months the VOKK publishes a newsletter about children and cancer, and this newsletter usually includes one or two pages on Fanconi anemia. ♦

## Fanconi Anemia in Argentina

by Cesar Lucero

The Fanconi Anemia Association (AAAF) began in October of 1997.

Because of Argentina’s economic situation at this time, it has become impossible to ask for any monetary contribution in our country. However, we provide translation help for non-English and non-French speakers who attend medical meetings in Europe. We also assist FA research by bringing FA bone marrow, blood, and cancer tumor samples to Europe for research by Dr. Haguenaer (Paris) and by Dr. Joenje (The Netherlands). We also provide information to physicians from Uruguay, and we send oxymetholone to Spain. ♦

## Italian Association for Fanconi Anemia Research (AIRFA)

by Giovanni Pagano

Established in 1989, AIRFA is aimed at fostering research and public information on FA, as well as providing FA families with reciprocal contacts and support. AIRFA has contributed to the Italian FA Registry, and to a number of investigations of Italian and international researchers. An ongoing European Commission-supported project on FA and other genetic diseases is coordinated and managed by AIRFA. ♦

# The Deutsche Fanconi-Anaemie-Hilfe

by Ralf Dietrich

The Deutsche Fanconi-Anaemie-Hilfe was founded in 1988 and incorporated in 1990. Presently, we have contact with 120 FA families in Germany and an additional 30 FA families in Switzerland, Denmark, Norway and Russia. No FA support groups exist in these countries. In Germany there are 5 local FA offices that have board members and volunteers, and one central office that has an executive director and family support coordinator. The central office is located in Unna-Sidinghausen. Most of the families we know personally through visits at home or the hospital and through contact by phone, letters or e-mail.

Each year our organization holds a 3-4 day meeting that brings together families, physicians, and scientists, with more than 100 participants from different countries. The program contains a lot of information and fun for everybody,

a special research workshop, and awards to FA scientists who have had important successes in FA research and treatment.

Our Newsletter *FA-Bote* is published one to two times a year. We are in close contact with other FA support groups worldwide and participate whenever possible in their family and scientific meetings. Representatives of the Deutsche Fanconi-Anaemie-Hilfe give lectures about Fanconi anemia at universities, hospitals or in the home towns of families who want to inform the public. The organization also helps those families who are interested in raising funds.

In the past 10 years the Deutsche Fanconi-Anaemie-Hilfe has raised about 1 million German marks and has been able to support the purchase of important testing equipment, as well as to help cover some personnel costs and fees at the Universities of

Amsterdam and Würzburg. The Deutsche Fanconi-Anaemie-Hilfe has a very close collaboration with different FA scientists worldwide and has aided some in obtaining blood, skin, bone marrow and tumor samples from FA patients in order to establish FA cell banks for research. Another accomplishment is the development of a special computer program that tracks a patient's blood counts before, during, and after treatment with androgens.

The main future goals of the Deutsche Fanconi-Anaemie-Hilfe are to raise more funds to support more intensive research into the prevention and treatment of cancer and leukemia in FA patients. To visit our homepage (German language only), type <http://www.fanconi.de>. For calls, dial 011-49-2308-2111 (best time 8:00 a.m. to 3:00 p.m. US east coast time). ♦

## News from the French Fanconi Association

by Sylvette Silverston

The AFMF (French Fanconi Association) was founded in 1990 through the efforts of several families whose children were affected by this serious disease. The purposes of the Association are to assist families through a newsletter published three or four times a year, family support, and through raising funds for medical research. The Association collects donations from families, their acquaintances and companies, and finances research programs in France and abroad. Managers of the association are all volunteers concerned about this disease because of their children.

The Association counts 50 members in France. Professor Eliane Gluckman at Hôpital Saint-Louis in Paris follows most of the patients. This hospital is the most important clinical center in France for Fanconi patients. We are also connected with the other Fanconi associations (American, Italian, Argentine, German and Dutch). Last year, we attended the Scientific Symposium in Amsterdam that was organized by the American association, FARE. ♦

### 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.



# FUNDRAISING

## Fundraising for FA Research

by *Connie Schenone*

I planned a dinner dance. Hardest fundraiser I ever did, but in the end it was worth all the effort. I had a goal to raise at least \$10,000, and I exceeded that goal and raised much more. Of course, there were expenses to cover, but in the end I raised well over \$10,000 for FA research.

It took one whole year of planning and preparing, letter writing, many phone calls, and some help from my friends. Picked out a great place for a dance. Got a famous '50s band to play. Sold the tickets for \$100.00 a person. Tried to get many big companies to sponsor tables of 10. Had to guarantee the place a head count of 200. Got about 240 people to attend. Unbelievable! Had to figure out—other than selling tickets—how else to raise extra funds for expenses and the Fund. So I decided to do an auction and to make a journal. I had businesses buy advertisements in this journal, which was then given to those who

purchased tickets. The auction involved prizes, so we had to get many gift items donated from merchants.

Most important, I would like to say that fundraising comes from the heart. If you want to do a fundraiser, it's because you have a true cause (you want to help). My true cause is my son, Craig, and all his brothers and sisters around the world who have FA. I was determined to raise as much as I could. I gave it my best shot, and it worked out. If you are fundraising and your heart isn't in it, you will give up or get discouraged. But just look into your child's face. Can you quit? Can you honestly give up? My answer is NO WAY! What's your answer?

If you have any questions about fundraising and if you need help, I'm loaded with ideas. Call me at (631) 298-9301. ♦



*The Schenone boys at the fundraiser. Left to right: Jared, 5 1/2; Joe, 15; Craig, 10.*

## Credit Card Donations

Credit card donations can be made to the FA Research Fund over the internet. Look for the PayPal button below the Donations line on the home page of the FARF web site ([www.fanconi.org](http://www.fanconi.org)). ♦

## Challenge to FA Families: Harold & Arlene Schnitzer CARE Foundation

The Fall 2001 *FA Family Newsletter* carried an article on the recent grant by the FA Research Fund to fund the International FA Transcription Consortium. Grover Bagby, Jr, MD, from the Cancer Institute of the Oregon Health & Sciences University, is the Principal Investigator for this project, the purpose of which is to study the causes of bone marrow failure and leukemia in FA. Transplant centers at the University of Minnesota; Children's Hospital,

Cincinnati; Capetown, South Africa; Curitiba, Brazil; and St. Jude's Hospital, Memphis, are participating in this project. The FA Research Fund, through the efforts of Lynn and Dave Frohnmayer, wrote a grant request to the Harold & Arlene Schnitzer CARE Foundation of Portland, Oregon to fund part of this project. The CARE Foundation awarded \$60,000 to the Fund for this purpose and has challenged FA families to raise a corresponding

\$60,000. We are asking you to help us meet this challenge. Through the generous assistance of three FA families, we have already reached almost one-half of our goal. Please urge your friends and families to help in this very important effort by donating to the FA Research Fund and noting on the check that the funds are for the CARE Foundation Challenge. ♦

## Golf Classic and Auction Raises over \$15,000

by *Christie & Randy Kelley*

On October 27 and 28, 2001, the Hunter Kelley and Fanconi Anemia Golf Classic and Auction was held, raising over \$15,000. The event was modeled after last year's successful Costume Ball and Auction, but this time the event was followed by a golf tournament the next morning.

Following a video about FA and one celebrating Hunter's life, the 200 guests enjoyed the band and a buffet of wonderful hors d'oeuvres and desserts while they browsed the 75 items on display for the silent auction. The next morning, the golfers enjoyed a continental breakfast. With a beautiful Toyota Sequoia as a hole-in-one prize, they were anxious to get started. After the tournament, everyone enjoyed lunch, followed by the presentation of prizes for 1st, 2nd and 3rd places. Both days were filled with fun, and we were truly amazed at everyone's eagerness to participate in our fundraising efforts. With help from friends and the many local

business people who donated items for the auction, we were able to raise awareness about FA as well as funds for much-needed research. We encourage everyone to organize a fundraising effort. With your help,

our children and loved ones with FA may have the chance to lead a full and productive life. The feeling that you are making a difference in the fight against Fanconi anemia makes all your efforts worthwhile! ♦



*Hunter Kelley*

## A Thank You from the Executive Director

by *Mary Ellen Eiler*

I want to thank all of you for your exceptional fundraising efforts during 2001. To the great credit of FA families, the FA Research Fund raised more money than in years past (a 91% increase from 1998), and more FA families than ever donated funds or asked their families, friends and acquaintances to donate on their behalf. The increase in the number of families participating in fundraising is of particular importance because of the Fund's great reliance on the fundraising efforts of only one family, the Frohnmayers. By increasing the numbers of families who fundraise, we have begun to

place a more secure foundation under the Fund.

We continue to put the donations that you have made or raised to excellent use. During 2001, the Fund sponsored regional meetings in Massachusetts, North Carolina, and Maryland and increased the accessibility of the FA Family Meeting to families through the Family Scholarship Program. We will be continuing both efforts during 2002. We also sponsored a highly successful meeting of bone marrow transplanters last April in Chicago, funded over \$500,000 of research, translated the Handbook into Spanish, and held

another very successful Scientific Symposium. We are most pleased to be able to report that our accountants recently completed the Fund's audit for 2001 and reported that our administrative expenses continue to be low—at 7.95% for 2001, which is outstanding by any measure.

I know that you are all struggling with the day-to-day difficulty of living with Fanconi anemia. Despite that—and, no doubt, because of that—you have made the effort to raise funds. I am profoundly grateful for your commitment to help find a cure for this devastating disease. ♦

## Family Fundraising Efforts for the Past Six Months

From July 1 to December 31, 2001, families raised \$1,666,147. The Fund also received \$8,435 from the United Way and the Combined Federal Campaign. Our special thanks to all of you who have worked so hard to raise vitally important research dollars.

### **\$39,000 and up**

Dave & Lynn Frohnmayer  
Kevin & Lorraine McQueen

### **\$20,000 - \$29,999**

Bill & Jackie Lucarell

### **\$10,000 - \$19,999**

Andrew & Vicki Athens  
Laurie Strongin & Allen Goldberg  
Randy & Christie Kelley  
Bill & Connie Schenone

### **\$5,000 - \$9,999**

John & Audrey Barrow  
Joseph Chou  
Charles & Katy Hull  
Robert & Andrea Sacks

### **\$1,000 - \$4,999**

Mark & Linda Baumiller  
Chris & Susan Collins  
Brian & Margaret Curtis  
Bill & Pat Danks  
Marie & Antonio DiMercurio  
Ed & Janice Duffy  
Erik Kjos-Hanssen & Turid Frislad  
Jeff & Beth Janock  
Eric & Beth Losekamp  
Peggy & Gil McDaniel  
Jack & Lisa Nash  
John & Dianne Ploetz  
Erik & Lori Salo  
Linda & Robert Scullin  
Bryan & Karen Siebenthal  
Mark & Susan Trager  
Michael & Beth Vangel  
Marc & Sandi Weiner

### **Up to \$1,000**

Lily & Keith Baggett  
Roger & Sarah Baker  
Kelly Bennett  
Darryl Blecher & Diana Fitch  
Randy & Nancy Bloxom  
Joaquim & Joelle Carvalho  
James Colon  
Patience Curry  
Donna DellaRatta  
Carol & James Dillon  
Pat & Mary DiMarino  
Kim Elzinga  
Frederic Engel  
Ezat & Laila Faizyar  
Nancy Fena  
David & Mary Ann Fiaschetti  
Beatriz Goris  
Andrew & Jennifer Gough  
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Karen Steingarten  
Richard & Janice Thomas  
Melissa & Steve Turner  
Nanette Vannostran  
The Family of Lynn Welfare  
Gerald & Elizabeth Wisz

### **Use of Logo**

This is just a reminder to our FA families: please use our logo or letterhead only after you have consulted the staff of the FA Research Fund, and received their approval. This is necessary to be sure our messages are accurate and consistent. It also helps to avoid legal complications. We are happy to collaborate on fundraisers and mailings.

### **New Logo**

You may have noticed a new look to the newsletter in the form of our new logo. We would like to thank Funk & Associates of Eugene, Oregon, for donating many hours of work and talent toward the creation of this logo and for redesigning our stationery. We are extremely pleased with the artistry and updated look that this logo brings to our publications. ♦

## Family Meeting 2002

*continued from 1*

to be presented will include transplantation, endocrinology, gene therapy, orthopedics (for hand and thumb anomalies), and solid tumors.

Interspersed with the scientific presentations will be psychosocial sessions with Nancy Cincotta, MSW, whose role is so very important in helping all FA families cope with this diagnosis. The meeting also provides families with a chance to network with other FA families, have personal contact with the speakers, and relax.

The children's program will be supervised by Camp Sunshine volunteers and will keep the kids busy with numerous age-appropriate activities. The facility includes an indoor swimming pool, computer center, recreational room, and a playground.

Camp Sunshine accommodates 50 families in suites, which sleep 5 people. Additional lodging is available near Camp Sunshine. Registration will be on a first-come, first-served basis, so register early with Suzanne Lauck at the FA Research Fund. ♦

## Attending the FA Family Meeting

*continued from 10*

courage facing this disease is a wonder to behold.

Suzanne and the other staff members were extremely friendly, caring and helpful. The volunteers from Michigan were a godsend. Never in our seven years together have I seen my husband show any signs of letting a tear drop. But it was emotional to say thank you and goodbye to the teenagers who took care of our kids so we could attend the seminars and go to the extra activities offered to us. We thank the volunteers so very much for their time and their patience with our children.

The doctors and the researchers were very informative and gave us a lot to think about when we got home. Thank God we did get the chance to go to the Family Meeting because, as soon as we got home, we had to make a lot of decisions right away about Clay because his counts were dropping.

We look forward to seeing everyone again this year, making more friends and hearing the results of the research advances achieved by the doctors and the researchers in this past year. ♦



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# The Fourteenth Annual International FA Scientific Symposium

## DECEMBER 2-5, 2002

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