FA family newsletter

#18 A Semi-annual Newsletter on Fanconi Anemia for Affected Families, Caring Physicians and Research Scientists Summer, 1995

Families Share Hopes and Concerns and Have Fun at Camp Sunshine Meeting

"It's a place that you can't find anywhere else." Those words, from one family's evaluation, express the overwhelming enthusiasm of participants who attended the fifth annual A family meeting.

For the third straight year, the generosity of Larry and Anna Gould and their dedicated staff and volunteers permitted us to gather at Lake Sebago, Maine. Ideal Camp Sunshine spring weather enhanced a full range of outdoor activities for adults and children alike. Leading medical researchers gave latest results in basic research and therapy. Results are most heartening (see *Scientific Supplement*).

Fifty-five families from seven nations shared hopes, concerns, and information, and developed bonds of caring. Thanks to the thoughtful foresight of the Goulds, families with demanding work or school commitments met for a "weekender" from May 19-21, where intensive medical research and group support sessions were accompanied by family recreation opportunities. Numerous families extended their visit at this wonderful site into or through the following week.

continued on p. 6

Masquerade Party at Camp Sunshine

HIGHLIGHTS

Gene Therapy Begins for FA-C Patient

In April, 1995, researchers at the National Institutes of Health (NIH) received final approval from the Food and Drug Administration (FDA) to conduct a gene therapy trial for a small group of FA patients in complementation group C. On May 26, 1995, the first FA patient ever to receive gene therapy began this trial. (See Family News, *Derek DaRosa Undergoes Gene Therapy!* p. 5 for a personal account). It is far too early to assess the outcome for this one patient. Early indicators are positive, and show that the normal gene is present in some cells. Subsequent tests will determine the percentage and type of cell carrying the FA-C gene, and the effect of gene therapy on this patient's blood production.

Researchers Johnson Liu, MD and Christopher Walsh, MD, received approval to treat six FA patients initially. To be considered for this trial, a patient must

continued on p. 7

MEDICAL NEWS

New Method May Improve Transplantation Odds for FA Patients without Matched Sibling Donors

John Wagner, MD, University of Minnesota Bone Marrow Transplant Program, has developed a new methodology to transplant patients who lack a matched sibling donor. His method of partial T cell depletion has been used with impressive results on approximately 20 patients with inborn (genetic) errors of metabolism and 23 patients with leukemia.

Of 43 patients, two failed to achieve a sustained engraftment. One patient developed grade III graft vs host Disease (GVHD) 'ue to an inability to tolerate yclosporin, a drug used to fight GVHD and maintain an engraftment. The remaining patients have engrafted with grade II or less GVHD. Wagner writes "with low rates of GVHD, we have observed early discharges from the hospital (average 25 days) and high rates of survival (78%)."

Wagner uses a method called counterflow elutriation. Donor marrow cells are placed in a machine called an elutriator and sorted according to their size and density, and therefore, researchers believe, according to cell type and function. Stem cells are injected immediately into the transplant patient. Remaining cells are put into a CellPro column, which collects additional stem cells. These ells, plus a certain percentage of T ells, are then given to the patient.

Some T cells are necessary for engraftment and may have an anti-leukemic effect. But too many T cells, especially from an unrelated or mismatched donor, often cause serious GVHD. Through years of animal studies and preliminary human trials, Wagner believes that his methodology can overcome two lethal complications in some patients with unrelated and one-antigen mismatched donors: lack of engraftment and fatal GVHD.

In early 1995, two FA patients were transplanted at the University of Minnesota using this technique. Both patients had one-antigen mismatched, unrelated donors. The first patient, age 22, had leukemia at the time of transplant. She engrafted early and six months post transplant has not developed GVHD. The second patient engrafted on day 15, but lost her graft soon thereafter. She died following a second transplant.

Wagner plans to transplant an additional 25 FA patients using counterflow elutriation. Depending upon the outcomes, his protocol may be altered.

For more information see *Scientific Supplement*.

Workshop on High Risk Transplants for FA Patients

Dr. John Wagner, University of Minnesota, will convene a one day workshop this fall on unrelated and/or mismatched donor transplants for Fanconi anemia patients. Transplant experts from large centers which have had extensive experience in performing these transplants will attend. The purpose of this workshop is to identify 1) the optimal timing of bone marrow transplants and 2) the treatment package that optimizes the success of unrelated donor transplants. Issues to be discussed will include: preparative therapies, use of T cell depletion, use of unrelated umbilical cord blood, and pre and post-immune suppressive therapy to reduce the risk of GVHD and graft failure. Wagner hopes that a common treatment protocol among several centers will allow more rapid identification of the best approach.

This workshop will be held in Minneapolis on September 18. It is co-sponsored by the Fanconi Anemia Research Fund, Inc. and the National Marrow Donor Program.

Video Tape Available

A videotape explaining Wagner's method of counterflow elutriation and the University of Minnesota's experience in transplanting FA patients with unrelated donors is available through the Fanconi Anemia Research Fund office. This video also gives families information on cord blood transplants and some guidance on choosing an appropriate transplant center. Write or call our office if you would like a copy.

Finding an Unrelated Donor

Fewer than 30% of FA patients considering bone marrow transplantation will have a matched donor among their family members. Many may want to search for an unrelated donor. The National Marrow Donor Program (NMDP) was created in 1986 to assist patients. As of July 1, 1995, the NMDP had 1,718,705 donors in its registry.

A search of the NMDP registry automatically accesses NMDP donors registered in the US, Israel, the Netherlands, and three of the German registries. By late summer, 1995, Sweden will also be included. NMDP has cooperative agreements which allow access to the national registries of Canada, Great Britain, France, Switzerland, Australia, Austria and Spain. Altogether, the NMDP has access to 90% of the registered bone marrow donors in the world.

THE SEARCH PROCESS

Much of the following information is from a flier published by the NMDP.

Step 1: The Preliminary Search

The preliminary search is free. NMDP conducts a computer file search to determine the possibility of finding a matched unrelated marrow donor for a patient in need of a transplant.

a. How to begin a Preliminary Search

Any physician may request a preliminary search on behalf of the patient. The physician may provide the necessary information by telephone to the NMDP Search Coordinating Unit at 1-800-526-3809, ext. 156 or by fax to 1-612-627-5810. The physician must provide the following information:

1. Patient's name (last, first and middle);
2. Patient's date of birth;
3. Patient's sex and race;
4. Patient's complete mailing address;
5. Patient's diagnosis;
6. Patient's disease status;
7. Previous history of blood transfusions;
8. Date of primary diagnosis;
9. Patient's HLA antigen typing;
10. The physician's name, mailing address and phone number.

b. Preliminary Search Results

Results of the preliminary search are normally returned to the requesting physician within 24 hours of the request. The report rank orders potential matched donors who are HLA-A, B and DR-typed and potential donors who are HLA-A and B-typed only. Potential donors are not identified by name or location. The results capture a "moment in time". Changes occur continuously as volunteer donors are added to or deleted from the registry and additional DR-typing information is added. Because of constant changes, the NMDP cannot guarantee a particular donor's availability. The preliminary search may be rerun as many times as necessary before a formal search is started.

Step 2: How to Proceed With the Search Process

After the preliminary search, the next step is to contact a participating NMDP-approved transplant center. NMDP's Office of Patient Advocacy (OPA) has prepared an Access Directory that provides detailed information on each participating network transplant center. The Access Directory is free and can be requested by calling OPA at 1-800-526-7809. **Only an NMDP network transplant center may begin the formal search process.**

Step 3. The Formal Search

The formal search is a multi-step process which starts when a

continued on p. 4

Muriel Lambert Receives NIH Grant

In June, 1995, Muriel W. Lambert, Ph.D., learned that the National Institutes of Health had just awarded her a grant to study the DNA repair defect in Fanconi anemia, complementation group A. She writes: "I want to thank the Fanconi Anemia Research Fund for the grant which it awarded me this past year which has allowed me to carry out the preliminary studies I needed in order to apply for this NIH grant. The research undertaken with these funds has resulted in two papers which I have submitted for publication."

Lambert is the fifth researcher to receive seed money from our research fund who has subsequently been awarded a prestigious, highly competitive NIH grant. Our research dollars have been multiplied many times over! We extend sincere congratulations to Dr. Lambert!

Unrelated Donor

continued from p. 2

patient begins working with a transplant center. Transplant centers charge between $600 and $3500 to formalize the search. This does not include costs of tissue-typing potential donors. The transplant center will select potentially matched donors for further compatibility testing. Donors will need to be DR-typed if they were only HLA-A and B-typed, or the donors will need confirmatory typing if they were HLA-A, B, and DR-typed. Finally, donors are DNA-typed to ensure that donor and patient are as compatible as possible, and that no errors have been made.

Before launching the formal search, you may wish to verify the availability of insurance coverage for this process. Coverage varies from plan to plan. Costs also vary considerably, depending upon the extent of tissue typing involved.

Step 4. Marrow Procurement

Once a donor has been confirmed as a match for the patient, the donor is counseled about the marrow harvesting process. If he or she is willing to participate and passes a complete physical exam, the transplant can be scheduled. Marrow is collected immediately prior to transplantation.

TIME FRAME

Searching for a potential unrelated donor can be a lengthy and expensive process. The average time from initiating a formal search until transplant is 4 1/2 months. There can be great variability from one patient to another.

Families should consult with their physician concerning timing of a search. Age may be a factor, since the likelihood of serious hematological problems such as leukemia often increases with age. Steadily declining blood counts, the need for therapy to increase blood production, and certainly the discovery of an abnormal clone could indicate the need to begin a search. Initiating a search does not commit a family to proceed with a bone marrow transplant, but does help evaluate the options available. **Patients should not wait until the development of leukemia to initiate a donor search.**

FA Office Goes Online

Yes, we have finally gotten a modem and our own e-mail account. Previously we used our Board President's account. Now you can contact the office directly at **fafund@rio.com**

Use of New Vaccine for Chicken Pox

During this year's annual family meeting, Dr. Nasrollah Shahidi spoke briefly about a new vaccine called famciclovir (Famvir®), which protects against the chicken pox virus. Families with questions or concerns about use of this vaccine and its potential risks and benefits to FA patients may refer to Dr. Shahidi's summary in this edition of the *Scientific Supplement*.

Stem Cell Bank Project for FA Families in Planning Stage

The Board of Directors and Scientific Advisory Board soon will select a major institution to operate a Stem Cell Bank (SCB) for FA patient families. Blood stem cells are the immature, self-renewing cells that generate all of the blood cells in the human body, including the cells responsible for the immune system.

The purpose of the SCB is to offer FA families an affordable means to harvest and preserve patient stem cells early in the course of the disease; that is, prior to decline in red and white cells and platelet production (pancytopenia), development of clonal abnormalities, or conversion to leukemia. The harvest may be taken through peripheral blood or bone marrow and cryopreserved (frozen). Patients or their parents may authorize withdrawals from their "stem cell account" for direct use in therapy, as a contribution to FA research, or both.

Families who are part of our Fund's Family Education and Support Network will be able to participate in the pilot project. They must present the SCB operator a documented FA diagnosis determined by DEB or MMC sensitivity testing.

The Board hopes to make its final decision regarding who will operate the SCB and a final project budget by late September. All families who receive this newsletter will receive an *FA Family Bulletin* describing the project, with start date, procedures, and costs.

FAMILY NEWS

Derek DaRosa Undergoes Gene Therapy!

by Susan DaRosa

Derek DaRosa is the first FA patient to receive gene therapy.

April 18, 1995—just another day to some, but to us and the other families dealing with FA it was a day we all had been waiting for. We finally got the news that gene transfer therapy was approved!! This news could not have come at a better time for us. Derek's counts had been giving us some problems and he was getting more irritated, argumentative and depressed. Derek was tired of dealing with this disease and not being able to do anything. He basically had given up hope of ever being able to play "normally" with his friends. He needed a boost. This news gave him (and us) something to look forward to.

We immediately started talking to Dr. Johnson Liu at NIH and started to make all the arrangements to go there. After a disappointing one-week delay, we arrived at NIH.

When Derek was getting the 12 tubes of blood drawn for all the different tests, it finally hit me that we were here at NIH and actually were getting ready to start the treatment. It took a while to sink in because everything happened so fast. But we adjusted quickly and were very eager to get started with the treatment.

The "plan of action" was thoroughly explained to us and we just took it one day at a time. First was starting an IV and doing a bone marrow biopsy. There was daily blood work and meeting lots of

continued on page 7

Catelyn Duffy Enjoys Disney World, Thanks to Brass Ring Society

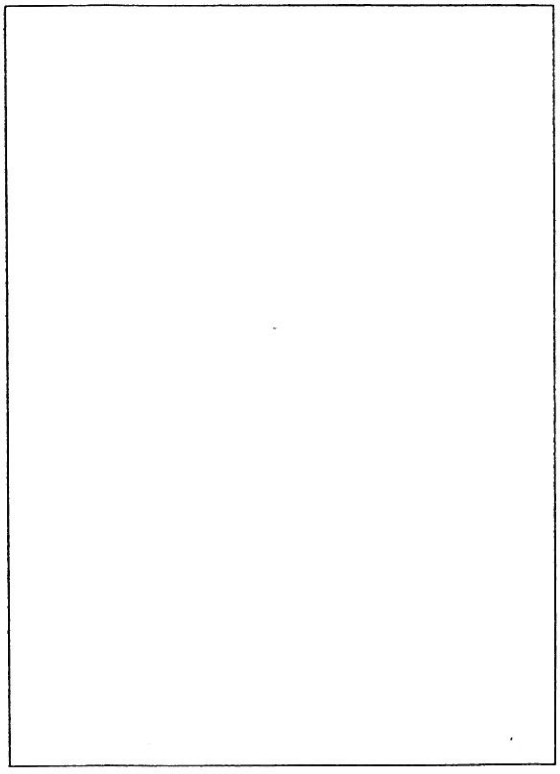
by Ed and Jan Duffy

We would like to tell you about our family's recent experience with the Brass Ring Society. In May the entire family spent a wonderful week in Walt Disney World, thanks to the efforts of Ray Esposito, President and Founder of The Brass Ring Society.

Our daughter Catelyn, age 7, had been asking all winter about the possibility of going to Disney World. Within days of speaking with Ray over the phone, we filled out an application which was quickly processed; three weeks later our application was approved, and we received an itinerary for one of several weeks that we had requested. We had a wonderful, fun-filled, stress-free week in Florida and would recommend this experience to any family. Our only regret was that the week we were away coincided with the Camp Sunshine trip. We had really wanted to visit again with all of the wonderful people in the group; we certainly hope to see you all at next year's meeting.

The Brass Ring Society will attempt to grant any reasonable request from a child affected with FA. We were told by Ray that the Disney World vacation is by far the most common request. The Society pays for the entire family to accompany the child whose dream trip is being fulfilled.

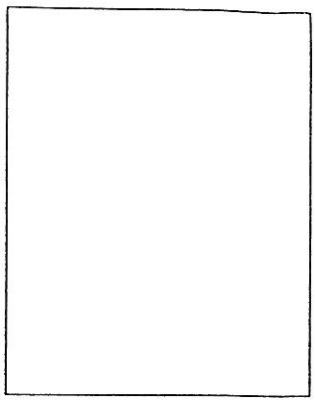
Ray Esposito and The Brass Ring Society can be contacted at: 551 East Semoran Blvd, #E-5, Fern Park, FL 32730, (800) 666-WISH.



Catelyn Duffy with brothers Matthew and Mikey

Chris Danchisko Gets His Wish

by Alison McClay (Chris' mother)



Chris and sister Alex

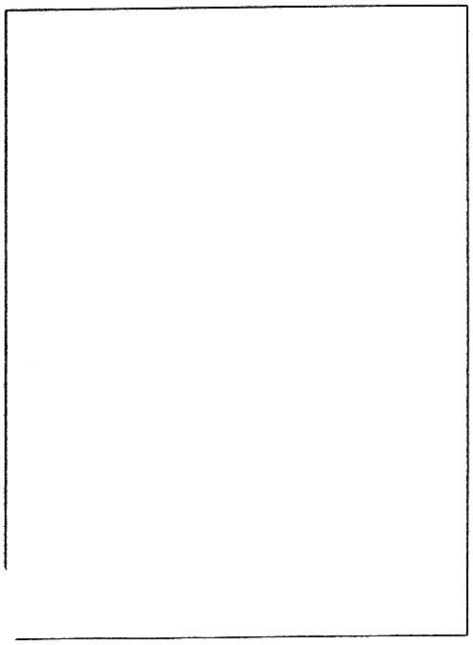
Christopher was born full term weighing 6 pounds and 18 1/2 inches long on March 24, 1981. His only physical abnormalities are his thumbs. He can use them, but they look "different". All his regular visits to his pediatrician were "normal". I am thankful I had 11and 1/2 years of not knowing about FA.

On August 12, 1992 our family went for our yearly examinations with a new doctor. After we returned home that fateful call came. The pediatrician said there was a problem with Chris' blood and we needed to return immediately to see the pediatric hematologist. We were still not overly concerned, thinking it must be a mistake in the lab. After our visit with the hematologist we were getting scared; we heard terms like blood counts, bone marrow, leukemia, and aplastic anemia. Okay, so what did this all mean? The next morning we were sent to the hospital for a bone marrow biopsy and aspiration. Hearing "it's not leukemia" sounded like a godsend. Little did we know it was worse. We were told horror stories about Chris possibly having 6 months to live. There was a mention of another horrible disease called Fanconi anemia. Tests done at Mount Sinai Hospital in New York confirmed our worst nightmare. How we got through those months of fear, anger, and sadness can only be answered by God.

Chris is now 14 years old. He is currently taking 25 mg of oxymetholone daily and 5 mg of prednisone every other day. His blood counts are pretty good at 150,000 platelets, 13 to 15 grams of hemoglobin and 2 to 2.3 white count.

Chris has a passion for music, sports, and now girls. In December he had a wish granted from the Mary Lyons Foundation. He wished for a Selmer Mark VI Tenor Saxophone.

Little did we know this instrument was a collectors' item and very difficult, not to mention expensive, to obtain. Two wonderful people from the fund, Belinda and Jerry, researched and were able to locate the Selmer. For a child who rarely smiles, you should have seen his face light up. He has a natural ability to play; he auditioned for and was accepted for the Central New Jersey Band. He also performed in his school concert and had a solo. We could not be more proud of him for his dedication and achievement. He never has to be told to practice, which shows his love for music. He has since purchased a set of drums and will soon be a one-man band. We truly believe one day Chris will be well known for his music and not his disease.



Susan and Christian Collins rest after lunch

Camp Sunshine

continued from front page

Children and young adults (and even older ones!) were captivated by magic shows, acrobatics, heroically sung karaoke, arts and crafts, campfires and the development of friendships. Both formal and informal sessions produced honest and productive family and group conversations.

"I leave here with hope," wrote one parent of a newly diagnosed FA patient, who deeply appreciated the opportunity to overcome the devastating isolation that affects FA families. "We needed very much to be here with the group this year, perhaps more than we thought," said the family of an inspirational child whose recent death is a source of continuing grief for us all.

The superior evaluations of the program, location, and staff and volunteer counselors of Camp Sunshine reflect great credit on all who helped make this experience possible. One evaluator put it in a nutshell: "My son says Camp Sunshine is the only good thing that has happened to him because of FA".

Thanks again to all who made this experience a reality.

Derek DaRosa

continued from previous page

ople. On May 22, the catheter was inserted in his groin, and apheresis began. During the apheresis (when they collect the stem cells they will later correct) Derek got to watch movies and basically was treated like royalty. He loved that part of the treatment. The apheresis continued for three days and Derek had to stay in the hospital because of the catheter. I wanted to stay with him, but Derek didn't want me to. I think he wanted to be away from "overprotective" mom for awhile.

On May 26, Derek got his corrected cells back. I expected there to be more to it but it was just a big syringe filled with cells. It was put into his IV. The procedure took only 10 to 15 minutes. We waited for an hour or so, to watch for a reaction, then left to go sightseeing. This same procedure was repeated the next day and he was done, so we got in some more sightseeing.

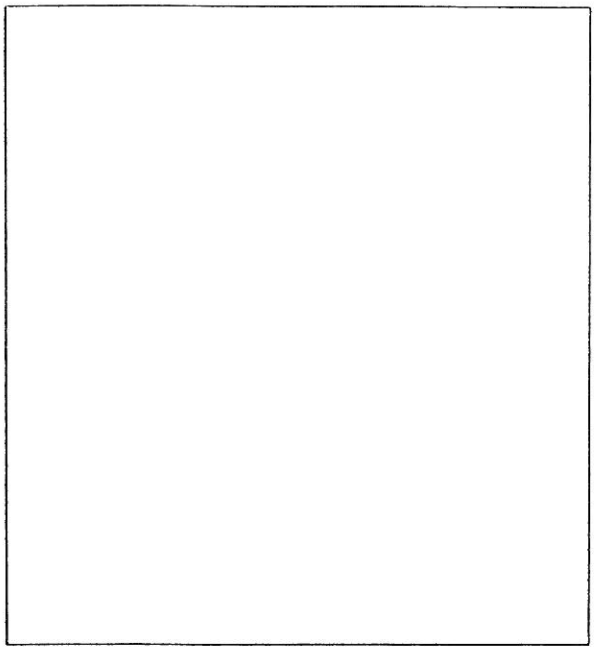
We felt that the whole treatment was really easy. The worst part was getting the IV in. Once that was done, everything went smoothly. Everything was done through the IV so he wasn't being poked all the time (except for the five days of G-CSF shots, but I had been giving him those for the past year so he was used to that poke).

Derek watched lots of movies and we worked on his schoolwork. We also spent a lot of quality family time together. It was great to be able to stay at The Children's Inn and not have to worry about anything. At the Inn each family does its own housekeeping, cooking, cleaning, everything. So we were able to keep our own routines from home. All the families who stay there are going through similar things. They know how you feel. I know Derek enjoyed being around kids who were also taking G-CSF and getting transfusions and not being able to play contact sports. They understood each other. They would get together and talk and compare notes about surgeries and medications. Derek was happy the whole time we were there and we were able to relax and take time for ourselves. Derek even had the nurses fighting over who would take care of him that day.

Derek, his father and I are looking forward to the next round of treatment in August. It will be the exact same treatment as he just went through.

If anyone wants to talk more or has questions, please call us at (408) 265-5972. If we aren't at home please leave a message and we will call back.

The DaRosas
Josh, Susan, and Derek (of course)



Derek receiving corrected cells

Gene Therapy

continued from front page

be in complementation group C and meet specific criteria (see Walsh's article in the *Scientific Supplement.*)

Patients will be treated with a hematopoietic growth factor called G-CSF (granulocyte-colony stimulating factor) for one week, to increase the number of stem cells in the peripheral blood. Blood cells will then be removed by a process called apheresis. The cells are treated in a test tube using a special cell-penetrating "vector" containing the normal FA-C gene. The corrected cells are returned to the patient. This process will be repeated four times over a year-long period.

Studies in the NIH laboratory suggest that Fanconi anemia may be a good candidate disease for gene therapy. Cells containing the normal FA gene grow better than uncorrected cells and therefore could have a competitive advantage. However, this first trial is experimental and risks are not yet fully understood. Initially, patients will be added with great caution and selectivity. If this trial proves successful, criteria for treatment will be expanded.

Shawn Philips

Vicki Phillips called in early June with the tragic news that her beloved son, Shawn, had died of complications of leukemia. She sent poems and newspaper articles, and asked that Shawn be remembered in our newsletter.

Shawn died on June 12, 1995 at the age of 24. He and Vicki attended several family meetings and were active in our support group. Those of us who were fortunate enough to know Shawn greatly appreciated his gentle nature, good sense of humor, and loving concern for others. He was respected and loved by many.

Shawn learned that he had leukemia in late 1994. In a thoughtful letter following his diagnosis, he talked about the wonderful things he had been able to do in his life, his genuine love of his work as a producer for radio station WQBK-AM in Albany, New York, and the many experiences he still hoped to enjoy. Shawn wrote:

"I was watching a Rick Reynolds Broadway show: 'Only the Truth is Funny'. He talked about the best way to determine if you are happy with your life. What would you do if you were presented with the following option: You are holding a coin. If you flip the coin, one of two things will happen. If it lands on one side, you get everything you've ever wanted in your entire life. Perfect health, money, love, etc. If it lands on the other side, you die instantly. Poof! You're gone. The question is, given the current state of your life, would you flip that coin? Take that chance? My answer is no. That's the best way I can describe how I feel."

Shawn lived his 24 years to the fullest. He enjoyed a very special, extremely close relationship with his mother, Vicki. His presence will be missed by so many.

To my friend, Vicki,

The child you nestled in your arms
So lovingly, and with such care,
Protecting him from all the harms
That might have "touched a hair".

All through his life you tended
To his needs and aspirations,
Every thought you comprehended - -
And moved with all its variations.

Too soon, your hands were tied - -
Reluctantly, you gave him to another;
Someone above, who would decide
That he no longer needed a mother.

In your place, the Almighty now cares
For the one you have loved so well.
Believe that His love also compares
To yours and in peace he will dwell.

When love is very deep,
Pain is unbearable;
A lost love will reap
Pain that is incomparable.

Together you have shared so much.
As his mother, you, who knew him best - -
His moods, sensitivities and touch - -
Now must let him go to his eternal rest.

God has called him home again,
To be forever in His loving care.
Mercifully, He took away his pain,
And bades us all not to despair.

While we sorrow, yet must we cope:
Only with love remember the Past,
And the Present with a fervent hope
That the Future holds peace, at last.

Dorothy Totten
June 21, 1995

Shawn D. Phillips Memorial Scholarship Fund for Radio Broadcasting

In Loving Memory of Shawn D. Phillips who was a producer at WQBKAM 1300 for the past three years, a scholarship fund has been set up to aid people interested in radio broadcasting.

Please make checks payable to: Shawn D. Phillips Memorial Scholarship Fund
Mail to: c/o 476 Ontario Street
Albany, NY 12208

Adults with FA Share Information

The FA Research Fund recently mailed a questionnaire to 30 adults with Fanconi anemia. Eighteen questionnaires were returned to our office, copied and mailed to everyone completing a questionnaire. We hope to encourage communication between our adult members, address their concerns through newsletter articles, and establish e-mail access to the Fund and to other adult patients.

Five adults with FA attended our recent Family Meeting at Camp Sunshine. Elizabeth Claypool, 31, spoke to a parents' support group about her experiences with FA. Please see her letter on this page. Parents were enthusiastic, and requested a panel discussion with additional FA adult attendees next year.

Please contact our office if you wish to fill out an adult questionnaire, receive information from others, and be a part of this informal network of FA patients.

Adult with FA Enjoys Camp Sunshine

by Elizabeth Claypool

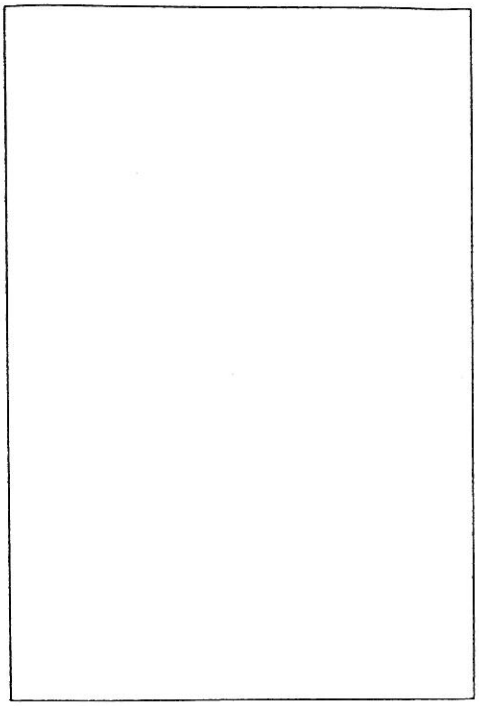
I hope everyone who attended the annual FA Conference at Camp Sunshine in Maine returned home safe and sound (my dog, Wiskers and I did!!). We missed those who were unable to attend!

It was nice to catch up with old friends and meet new people—for me, especially adults with FA.

There were many parents of children with FA with whom I had the opportunity to visit. Wow!! And so many questions in one of the parent support groups (as well as individually)!! I hope that my life experiences growing up, and as an adult, were helpful. I truly enjoyed meeting many people.

For those I did not have a chance to talk to or who were unable to attend, I have been living with FA since my diagnosis in 1970, and I am 31 years old. As I told many people, it hasn't been an easy road, but with much prayer, perseverance, and a positive attitude, I have done many things in my life. What I told people was that one needs to take things as they come, deal with them, AND GO ON!! This can sometimes be a very hard and scary thing to do, for those with FA and family members. If you waste time worrying about lots of things, you might miss out on the more important moments of life!! Deal with what you can today. Tomorrow will come soon enough. Leave the past in the past; you cannot change it. Don't give up on hope, strive to continue forward. And last, but not least, don't dwell on the disappointing moments. I guess you can say that this is the philosophy of life I strive to live by.

Until next time... have a safe and fun rest-of-the-year!!



Elizabeth Claypool and Wiskers rest at Camp Sunshine

In Loving Memory

Katie Danks 1/95
Debbie Dobrosky 1/95
Leslie Cramer 3/95
Bobby Lamb 6/95
Cole Parker 6/95
Shawn Phillips 6/95
Jamie Robertson 6/95
Donna Shimomura 6/95
David Russo 7/95
James Bradley 7/95

Families are always welcome to submit an article in memory of a cherished family member.

FUNDRAISING

Tiger Foundation Grants $75,000 for FA Research

The Tiger Foundation of New York has granted the FA Research Fund $75,000 for research: $25,000 now and $50,000 later if FA families raise a matching $50,000.

We are deeply grateful to Gerald Norris and the Tiger Board of Trustees for their continuing support. Between 1991 and 1993, Tiger provided our Fund $405,000 for FA research, $220,000 in the form of challenge grants. FA families met and exceeded these challenges. We will meet the challenge again!

Do you need help with your fundraising project? Call Leslie or Linda at the Fund office and see how simple it can be to answer the

continued on page 12

Research Funding Update

New Grants Awarded January 1994 - June 1995

Identifying and Cloning FA Genes	
Assessment of the Number of Major FA Genes Hans Joenje, Amsterdam, The Netherlands (2 years)	$132,020
FA Cell Repository and Molecular Studies Markus Grompe, Oregon	18,075
Identifying FA-A Using Mutant Rodent Cells Margaret Zdzienicka, Leiden, The Netherlands	25,000
Positional Cloning of the FA-A Gene (2 labs) Freerk Arwert, Amsterdam and Chris Mathew, London (1 year)	169,700
Isolation of FA Genes (non-C) by Functional Complementation Arleen Auerbach, New York	43,684
Understanding the Function of the FA Protein	
DNA Repair in FA Cells Muriel Lambert, New Jersey	29,740
Identifying Functional FA Protein Antibodies Maureen Hoatlin, Oregon (2 years)	120,030
Developing Effective Treatments	
Pilot Study of Interleukin 11 (platelet stimulator) Wayne Rackoff, Indiana	24,682
Total Project Support 1/1/94 - 6/30/95	**$562,931**

Slaters and LeRoux Combine Forces to Raise Funds

by Rene LeRoux

Our deepest thanks to Rene, Peg, Jeff and Debby for their hard work and significant contribution to our cause!

Recently, my wife Peg and I, together with Jeff and Debby Slater held a benefit dinner for our children Lauren and Nick. The dinner was a sit down semi-formal affair to which about 130 people came. We charged $30 per person. We had special invitations printed for free, and each family invited a certain number of people. We had hoped for an attendance of 200.

We had three profit centers for the event:

1) Ticket prices for the dinner.

continued on page 12

Incredible News from Denver!

by Teddi Matlack, mother of FA patient Ronnie

The Colorado lottery has a drawing every year where people can submit their losing scratch tickets at their local King Soopers grocery store. On the back of the tickets they write the name of their favorite charitable organization. Out of 1.1 million tickets, the ticket held by Dick and Judi Selke, grandparents of Ronnie Matlack, was chosen fourth. This drawing won $2,000 for Fanconi anemia research!

Family Fundraising Efforts

From January 1, 1995 through June 19, 1995, fifty-one families raised a total of $100,159. The FA Research Fund received an additional $5,070 from the Combined Federal Campaign and United Way which we were unable to attribute to specific families' efforts. Please let us know if your hard work is generating these contributions, so that we can thank you for your help.

Our ability to support scientific research, hold annual scientific meetings and disseminate information to families and physicians depends on our capacity to raise needed revenues. This year, we have the added incentive of a Tiger Challenge Grant. If you have helped before, please consider asking your donors for help once more. They have an investment in our progress; most will want to help again. If you have never raised funds for research, let us know how we can help you get started. If we all make an effort, we will reach our goals.

The amounts reported below were deposited in our account by June 19, 1995. Later deposits will be reported in the next newsletter. Our deepest thanks to each and every one of these families!

$50,000

Lynn & Dave Frohnmayer

$10,000

Carol & Jim Siniawski

$5,000 - 7,000

Pat & Bill Danks
Debby & Jeff Slater
Peg & Rene LeRoux

$1,000 - 5,000

Linda & Mark Baumiller
Tracey & Joseph DeMarco
Dick & Judi Selke
Deane Marchbein & Stuart Cohen
Sandy & Marc Weiner
Mai Byrne & Des Murnane
Therese & Terry Robertson
Fredi & Ron Norris
Phyllis Cafaro

$500 - 1000

Jackie & Bill Lucarell
Lorraine & Kevin O'Connor
Diane & Matt Senatore
Alison & Steve McClay
Linda & Robert Scullin

$100 - 500

Leardon Keleher
Cecelia Meloling
Ida Hodge
Alice & Bob Nicholson
Sandy & Eddie Allen
Iris & Neil Frank
Myra & Mike Lewis
Barbara & John Miller
Susan & Mark Trager
Ridgely & Durant Worthy
Sarah Baker
Susan Combs
Karilyn & John Kelson
Mary & Pat DiMarino
Pat & Ken Rau
Linda & Martin Sankey

Up to $100

Griff & Cecilia Morgan
Nancy Dobrosky
Vicki & Andrew Athens
Virginia & Louis Napoles
Lisa & Jack Nash
Maureen & Glenn Russo
Carol & James Dillon
April & Don Benton
Chris & Susan Collins
Ann & Nathan Eckstadt
Karen & Gene McDaniel
Robin Paulson
Forrest Engel
Jennifer & Robert Kiesel
Marie & William Schenone

We Honor Our Grantors and Benefactors January 1994 - June 1995

Amgen
The Donald R. Barker Foundation
The Cafaro Company
The Chiles Foundation
Collins Medical Trust
Georgia Pacific Corporation
Samuel S. Johnson Foundation
Phil Knight
Meyer Memorial Trust
Oregon Community Foundation:
The Summerville Fund
Bowerman Family Charitable Fund
Edwin E. & June E. Cone Fund
Kenneth & Colleen Lewis Fund
L.L. Stewart Fund
R.B. Pamplin
F.E. Stewart
Tiger Foundation
Rose E. Tucker Memorial Trust

Slaters and LeRoux

continued from page 10

2) Dinner program. We sold ads for $100, $150 and $200 for a full page.

3) Auction after the dinner. We went to local stores and shopping malls asking for a gift certificate or an item to auction. We divided items into two categories: live auction for items worth over $50 and a silent auction for items less than $50. A friend agreed to be the auctioneer; a local news anchor was the emcee for the dinner.

This event raised over $10,000 (auction, $4,000; program, $3,000; tickets, $3,000).

We each had an area of responsibility. My wife was responsible for the ads, Debby Slater for the auction items, and I got the restaurant, food and tickets donated. We all helped with each others' projects. Peg and Debby decorated the room and we all worked on the clean-up crew afterward.

We showed the FA movie on a big-screen TV that Jeff borrowed for the night. We had three guest speakers talk about various topics. The event lasted three hours.

We did all this work in about eight weeks. It gets a little crazy when you do not have a lot of time, but basically, it was a lot of fun.

Having two families involved made it nice. It made it seem like we were not alone. Having the people come was heartwarming; probably their support meant as much as the donations.

Tiger Foundation

continued from page 10

Tiger Challenge. Donation return envelopes and mailing services are available. **Reach us on our new toll-free FA Family Outreach line at 1-800-828-4891.**

Meanwhile, be sure to tell your family and friends they may **double** their gift by making their check payable to the FA Research Fund, and writing "Tiger" on the check memo line!

Making new friends at Camp Sunshine

Message from the Editors

Some years ago in this space, we reflected with awe on the energy that infused our first FA family meeting. We hadn't known that anyone would actually care to attend.

From that surprisingly successful beginning in Washington D.C. in 1991, many benefits have arisen: deeply caring family friendships; substantial increases in research-related fundraising; and shared grief from the loss of beloved family members.

We also have developed a realization that **our small number of determined families can change the course of medical science!**

Fanconi Anemia Research Fund, Inc.
1902 Jefferson St., #2
Eugene, OR 97405
(503) 687-4658
FAX (503) 687-0548
e-mail: fafund@rio.com

Executive Coordinator:
Linda M. DeSpain

Family Support Coordinator:
Lynn Frohnmayer

Administrative Assistant:
Leslie Roy

Newsletter Published by:
Fanconi Anemia Research Fund, Inc.

Medical Advisor for Newsletter
N.T. Shahidi, MD

Editors:
Dave & Lynn Frohnmayer and Joyce Owen

Layout and design:
Tanya Harvey

Printing donated by:
Shelton-Turnbull Printers

Distribution Support donated by:
Amgen, Incorporated
The Edwin & June Cone Fund of the Oregon Community Foundation