FANCONI ANEMIA RESEARCH FUND, INC

FAMILY NEWSLETTER

#19 A Semi-annual Newsletter on Fanconi Anemia for Families, Physicians and Research Scientists Winter, 1996

Some Highlights of the Seventh Annual FA Scientific Symposium

More than 100 scientists from around the world attended the Seventh Annual FA Scientific Symposium at Boston Children's Hospital on November 9-11, 1995. The National Institutes of Health, Heart, Lung and Blood Institute co-sponsored the meeting, along with The Collins Medical Trust, Sandoz Foundation, The Samuel S. Johnson Foundation, Louisiana Pacific Foundation, and the Rose E. Tucker Charitable Trust. Summaries of the presentations are published in the *Scientific Supplement* to this newsletter. Here are a few of the highlights.

• Promising results of first gene therapy trial

Johnson Liu reported on the first FA patient to undergo gene therapy. He is doing quite well, with good blood counts (see related article on page 2). The normal FA-C gene is present in one out of 10,000 mononuclear cells. It is too soon to tell if the corrected cells will have a competitive growth advantage (as hoped) over the defective cells.

• Two more FA genes localized

Using linkage analysis, the labs of Fré Arwert and Chris Mathew have identified the precise location of the FA-A gene near the tip of the long arm of chromosome 16 (16q24.3), and should very soon have the gene isolated and cloned. The FA-A gene is defective in two-thirds of European FA patients. Markus Grompe, using microcell-mediated chromosome transfer, has established that the FA-D gene is on the short arm of chromosome 3 (3p).

• More complementation groups discovered

Hans Joenje has found several additional complementation groups among European FA patients, bringing the total number to 7 or 8, but he is not yet positive that all of

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HIGHLIGHTS

Annual Family Meeting Returns to Camp Sunshine May 16-21, 1996

Mark your calendars. Budget for airfare. Camp Sunshine at beautiful Point Sebago, Maine again will be the site for our annual FA Family Meeting. Anna and Larry Gould have invited us back and we are delighted to accept. The challenge is on: can we surpass last year's record of 57 families from seven countries?

Camp Sunshine Program Administrator Tim Rabine and FARF staff are ready to fine-tune the program, but they need to know your priorities. Be sure to note your top choices for scientific presentations and focus groups on the Early Registration card that you should have received by now. Indicate any special lodging needs you may have. Once the FARF staff receives your Early Registration card, Camp Sunshine will send a complete registration packet to your home address.

Camp check-in will start on Thursday, May 16 at 2:00 p.m., followed by the lively Welcome Dinner. Youth day camps and the adult education program will start

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MEDICAL NEWS

First FA Gene Therapy Patient Doing Well!

Susan DaRosa reports that her son Derek, the first FA patient to undergo gene therapy, is doing very well. His blood counts are good. He's had no nosebleeds, which were always a problem before. His energy level is much higher; he's always on the go; before he was always tired. His dose of oxymetholone has been decreased to 50 mg every other day. He is due to receive his fourth and final infusion of corrected stem cells in April.

BLOOD COUNTS:

Before gene therapy

white	4,700
Hgb	8.6
platelets	21,000

After third infusion

white	15,000
Hgb	13
platelets	46,000

While results so far are promising, it is still too soon to tell whether this therapy will be successful over a long period, and whether the corrected cells will have a growth advantage over the defective cells. See *Some Highlights of the Symposium* on the front page, and the article by Johnson Liu on page 15 of the *Scientific Supplement.* ◆

FA Stem Cell Bank Launched at Indiana University

Dr. David Williams, project supervisor for the FA Stem Cell Bank (SCB) at Indiana University, reports that within four weeks of the project launch date on December 15, 1995, five FA-C families had submitted requests to participate.

Stem cells are a precious resource for treatment and research. With a family's permission, blood not used for therapy will be made available to qualified FA researchers. An interdisciplinary advisory committee will set criteria for use of SCB cells in research. Members of the Stem Cell Project's research advisory committee (all MDs) are Grover Bagby, Oregon Health Sciences University (Hematology/Oncology), Blanche Alter, University of Texas-Galveston (Hematology/Oncology), Deane Marchbein, FARF Board of Directors and FA Family Representative, Malcolm Brenner, St. Jude Children's Hospital-Memphis (Gene Therapy), Johnson Liu, National Institutes of Health (Gene Therapy), and Richard Harris, Children's Hospital-Cincinnati (Bone Marrow Transplantation). ◆

New FA Treatment and Research Group for Northeastern US

Drs. Al Gillio, Arleen Auerbach, and Joseph Gertner announce the formation of the Fanconi Anemia Treatment and Research Group (FATRG). This cooperative group is committed to the comprehensive care and support of FA patients and their families in the tristate area of New York, New Jersey, and Connecticut.

Gillio will leave Sloan-Kettering Cancer Center on March 1 to establish the clinical hematologic arm of the group at Tomorrow's Children's Institute at Hackensack University Medical Center. The Hackensack University Medical Center is located approximately 20 minutes from midtown Manhattan. Routine care and new experimental therapies will be available, including unrelated bone marrow and cord blood transplants. Auerbach will continue to lead the research arm of this group at The Rockefeller University, but will also hold an appointment at Hackensack and participate in clinical activities there. Gertner of The New York Hospital will coordinate the endocrinologic evaluation of FA patients with specific emphasis on growth abnormalities.

FA family education and support sessions will be offered to patients at Hackensack, including updates on progress in all aspects of FA research. For information regarding the program or appointments call Tomorrow's Children's Institute at 201-996-5437 or Auerbach at 212-327-7533. ◆

Symposium Highlights

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them represent separate genes. There are at least 3 (FA-C, FA-A, and FA-D have been mapped to three separate chromosomal locations), and there is evidence for 7 or 8. In his summary (see *Scientific Supplement*), he describes how complementation analysis is done.

• New developments in bone marrow transplantation

Richard Harris summarized results of BMT from various centers. His own results with matched sibling donors are extraordinary: 19 out of 21 are alive and fully engrafted, with no GVHD greater than grade I. He summarized an encouraging report in *Lancet* on cord blood transplants from related donors; engraftment was very high, GVHD very low. Hal Broxmeyer reported on the use of cord blood for transplant, as well as for gene therapy. John Wagner discussed unrelated BMT. The use of counterflow elutriation to remove donor T cells (which cause GVHD) is very effective in preventing GVHD. He discussed results of cord blood transplants using mismatched unrelated donors: three of four patients are alive with little GVHD. Cord blood is a promising source of stem cells for unrelated mismatched transplant, and should certainly be considered by anyone without a matched sibling donor. A multi-institution study is underway to determine an optimal protocol for unrelated mismatched transplant.

• Specific FA-C mutations determine severity of disease

Al Gillio and Arleen Auerbach report that patients with either of two specific mutations in the FA-C gene (IVS4 and mutations in exon 14) suffer from more severe disease than patients with another FA-C mutation, or patients with defects in other FA genes. They are more likely to have skeletal abnormalities, small head, and earlier onset of bone marrow failure and leukemia. All FA patients of Ashkenazi Jewish descent have the same severe mutation, IVS4, which has so far been found only in this group.

• New approaches to gene therapy

Robert Debs reported that liposomes can transfect all kinds of tissues, including both dividing and non-dividing cells, and can carry pieces of DNA of any size. Janet Lewis and Nasrollah Shahidi reported that the gene gun can deliver many copies of a gene rapidly; using this method they were able to transduce 6% of stem cells. Arun Srivastava described a new viral vector, a hybrid between adeno-associated virus (AAV) and parvovirus B19; the hybrid infects blood cells specifically. This vector integrates into the host DNA at a specific site, so it cannot interrupt (and inactivate) any essential genes. It should be useful in treating hemoglobin diseases, like sickle-cell anemia and β-thalassemia, as well as other blood diseases like FA.

• FA-C not due to cell cycle defect

Because FA cells treated with crosslinking agents (mitomycin-C or DEB) tend to accumulate in a certain phase of the cell cycle (phase G_2), it was thought that the fundamental FA defect might be a defect in regulation of the cell cycle. Comparing normal cells and FA-C cells, Michael Heinrich showed that normal cells treated with higher doses of crosslinking agents, so they had the same amount of DNA damage as the treated FA-C cells, also accumulate in G_2. In other words, it appears to be the level of DNA damage itself that leads to the G_2 accumulation, not a defect in the cell cycle.

• FA-C protein not DNA repair enzyme

Several labs report that the normal FA-C protein is found in the cytoplasm of the cell and not the nucleus (the expected location for a DNA repair enzyme). Hagop Youssoufian "tagged" the FA-C protein so it would go to the nucleus, and found that it no longer protected DNA from crosslink damage. He

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FA Families Chat at Scientific Symposium: Deane Marchbein and Beth, Roma (Mike's mother), and Mike Vangel.

Cord Blood: A Lifesaving Resource

At least four million children are born each year in the United States; the umbilical cords from most of them are discarded. However, preserving a newborn's cord blood can save the lives of patients with Fanconi anemia and other blood diseases. More and more expectant parents choose to donate their newborn's cord blood to registered blood banks for use in transplant therapy and research around the world.

Cord Blood for Transplants

Researchers have learned that cord blood may be a safer and more effective alternative to bone marrow for transplants. In 1988, the first cord blood transplant was performed in Paris, on a patient with FA. Since then, over 155 cord blood transplants have been performed worldwide.

Cord blood contains a rich supply of stem cells. Stem cells give rise to all other blood cells. While abundant in cord blood, and easy to collect with no risk or discomfort to the donor, stem cells later reside in the bone marrow where they are difficult to extract. Cord blood offers other advantages over bone marrow as well. Two of the greatest risks in bone marrow transplant are failure to engraft and graft vs host disease (GVHD). The rate of engraftment is high with cord blood, and the incidence and severity of GVHD are lower. In addition, cord blood is less likely to carry infectious agents, like viruses.

Cord blood collected from any healthy newborn may later be used to transplant a patient with FA, leukemia, or other blood disease. The cord blood may be banked by the family as insurance, in case that child later develops a blood disorder. For more specific information about the use of cord blood for transplant in FA, see the articles by Richard Harris and John Wagner on pages 10 and 11 of the *Scientific Supplement.*

Cord Blood for Gene Therapy

If expectant parents know in advance that their baby has FA, it might make sense to collect and store the baby's cord blood at delivery; it is possible that the cord blood could be used for gene therapy some time in the future. In current gene therapy, the patient's own stem cells are collected after treatment with G-CSF, which increases the number of stem cells circulating in the bloodstream. Then the stem cells are collected, treated to insert the normal gene, and infused back into the patient. It may be possible to use the patient's own frozen cord blood as a source of stem cells. This has already been attempted for another genetic blood disease. See the article by Hal Broxmeyer on page 12 of the *Scientific Supplement.*

Collection of Cord Blood

Cord blood collection is simple and painless. But plan early. Most banks prefer a 60-day advance notice. Cynthia Fisher of ViaCord, a private collection and storage company, finds that many insur-

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Cord Blood Banking Services

For Your Family Only

ViaCord Cord Blood Banking Service: Boston

Collects nationwide, has comprehensive information packets and video you may obtain without obligation. ViaCord banks cord blood at Hoxworth Blood Center, Cincinnati. Sarah Green, Client Services Representative, 800-998-4226.

University of Arizona Cord Blood Bank

Collects nationwide, collaborates with the International Cord Blood Foundation. Dr. David Harris, Director of Cord Blood Banking, 800-588-6377.

Hoxworth Blood Center, Children's Hospital Medical Center: Cincinnati

Collects nationwide, using ViaCord services; will send dedicated blood to the family anywhere in the US for therapeutic use. For more information call Dr. Richard Harris, Director of Bone Marrow Transplant Program, 513-559-4266.

Indiana University Riley Hospital for Children: Indianapolis

Collects cord blood for its FA patient families; for more information call Dr. Frank Smith, 317-274-3304.

Charges and warranties vary

Donating Cord Blood

International Cord Blood Foundation: San Francisco

Non-profit collaborator with the University of Arizona Cord Blood Bank. Raises funds to support collection and storage costs, provides education and donor recruitment services. 800-747-3319 or e-mail Larry Andreini at: LAndreini@aol.com.

New York Blood Center: New York City

Non-profit collaborator with Mt. Sinai Hospital, New York City. Baby must be delivered at Mt. Sinai. Make arrangements directly with Dr. Pablo Rubenstein, head of immunogenetics at the Center. 800-692-5663, press option 2-National Marrow Donor Program.

Milan Cord Blood Registry: Milan, Italy

39-2-55034053-5

Dusseldorf Cord Blood Registry: Dusseldorf, Germany

49-731-954300

You may locate additional cord blood banks by (1) talking with your physician, (2) calling the obstetric or transplant unit at your nearest major medical center, or (3) contacting the research services office at your state university's school of medicine.

Cord Blood

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ance companies and Medicaid will pay for cord blood collection when inherited or acquired conditions are indicated in a family. Cord blood banks vary in their scope of operation and fees charged. All will gladly respond to questions.

If you wish to learn more about how to bank or donate cord blood, costs of collection and storage, or FDA-approved blood testing and safety requirements, we suggest you research carefully by calling more than one of the resources listed on this page and the previous one. Then consult with your physician and your insurance company. The FA Research Fund will update its referral file regularly, so please give Leslie Roy or Linda DeSpain a call. Share your plans or experiences with them at 1-800-828-4891. ◆

Symposium Highlights

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suggests that the normal function of the FA-C protein is to prevent crosslink damage; it is not a DNA repair enzyme. Several labs are trying to isolate other proteins that bind to (and presumably work with) the FA-C protein. These may be the products of the other FA genes.

- **Damage done to FA cells by mitomycin C may result from oxidative damage rather than crosslinks**

Mark Kelley found that a ribosomal protein, S3, which repairs oxidative DNA damage, complements FA-A cells treated with MMC, restoring them to normal. He is testing whether the S3 protein can rescue other FA cells besides FA-A.

Donor Registries

Our last FA Family Newsletter included an article about bone marrow transplants which highlighted the services of the National Marrow Donor Program. Shortly thereafter, the American Bone Marrow Donor Registry contacted our office to ask that we inform our families, physicians and resource people of their services. Because the two registries offer different listings, you may wish to contact both to do a thorough search.

The American Bone Marrow Donor Registry
The Caitlin Raymond International Registry
The U. of Massachusetts Medical Center
55 Lake Avenue
Worcester, MA 01655
(508) 756-6444
(508) 752-1496 FAX

The National Marrow Donor Program
National Coordinating Center
3433 Broadway Street N.E., Suite 400
Minneapolis, MN 55413
(612) 627-5800
(800) 526-7809
(612) 627-5899 FAX

- **FA-C mouse models**

Both Markus Grompe and Manuel Buchwald have developed strains of mice with FA-C mutations. Surprisingly, these mice show no skeletal or blood abnormalities, but they have greatly reduced fertility. Cells from these mice are hypersensitive to DEB and MMC, confirming that they are indeed FA mice. Grover Bagby found that cells from Grompe's mice are very sensitive to γ-interferon. ◆

Researchers Testing Alternative Transplant Protocol

Fewer than 30% of patients in North America have an HLA-matched sibling. Graft failure and severe graft vs host disease [GVHD] substantially limit the success of alternative bone marrow transplants, those using mismatched or unrelated marrow.

In September, 1995, Dr. John Wagner convened a meeting of leaders in transplantation and research to discuss transplant outcomes in alternative donor transplants for FA patients. The meeting was held at National Marrow Donor Program (NMDP) headquarters. Many previously unreported cases were presented. Attendees expressed a strong commitment to develop a uniform protocol, in an effort to improve the outcome of alternative transplants for FA. The National Marrow Donor Program offered to compile trial and post-transplant data. The studies are now underway.

In addition to opening the way for patients who do not have matched sibling donors, the new protocol is designed to serve patients in an aggressive phase of their disease. Those with severe pancytopenia, myelodysplasia with persistent clonal chromosomal abnormalities, or leukemia may be eligible. For full patient eligibility criteria, see Wagner's article in the *Scientific Supplement*. The criteria are listed on page 18.

Participating researchers were Arleen Auerbach, The Rockefeller University; James Casper, Medical College of Wisconsin; Gaye Crooks, Children's Hospital of Los Angeles; Stella Davies and John Wagner, University of Minnesota; Joachim Deeg, Fred Hutchinson Cancer Research Center; Al Gillio, Memorial Sloan-Kettering Cancer Center; Eliane Gluckman, Hôpital St. Louis; Eva Guinan, Boston Children's Hospital; Richard Harris, Children's Hospital of Cincinnati; and Margreet van Weel, University of Leiden.

Financial and staff support for the day-long conference was provided by the NMDP and the Fanconi Anemia Research Fund, with a generous donation from Phyllis Cafaro. ◆

Back row: Joachim Deeg, James Casper, Eva Guinan, Arleen Auerbach, Margreet van Weel, John Wagner, Al Gillio; Front row: Lynn Frohnmayer, Stella Davies, Gaye Crooks, Eliane Gluckman, Richard Harris.

FAMILY NEWS

Henry Strongin Goldberg

By Laurie Strongin, married to Allen Goldberg

"Congratulations, it's a boy!" Those words, delivered to me and Allen at 8:51 a.m. on October 25, 1995 were the sweetest words I had ever heard. Our dream of having a baby and all the love and joy it would bring into our lives had been realized. And what a beautiful little boy he was.

Weighing in at five pounds even, Henry had exceeded our expectations. We had been told two weeks earlier, at 36 weeks gestation, that he had stopped growing and was likely to weigh somewhere around four pounds. I feared that he would need to be put in the intensive care nursery and, even worse, have to remain in the hospital beyond my stay. After two long weeks of close monitoring by sonogram, fetal monitor and non-stress tests, I delivered Henry by C-section and upon hearing his beautiful, innocent cry, experienced a sense of relief and exaltation unrivaled in my blessed life.

After a quick checkup, the attending doctors brought Henry to me and Allen, telling us everything looked just fine. Upon closer inspection, however, they noted that he had an extra thumb on his right hand, which, they assured us, could be removed by surgery quite easily. The thought of my little precious baby having surgery within a year of his life brought tears to my eyes. Although I had only known him for less than five minutes, I was madly in love with him and couldn't bear to think of him experiencing pain.

Within two hours, our world began to fall apart. The doctors heard a heart murmur and called a cardiologist in to examine Henry further. We were told he had a serious heart condition called Tetrology of Fallot and would need two surgeries within the first year of life—one to place a shunt in his heart and the other, open heart surgery, to do the definitive repair. Because of his thumb and the intrauterine growth retardation a geneticist would come to examine him further to see if all of these problems were related as part of a larger "syndrome".

I felt lost in a sea of fear and sadness. I wanted to hold Henry, but I was bedridden, recovering from surgery and he was in intensive care hooked up to heart monitors, oxygen and IV. Would I ever get to hold him in my arms? At 10:30 that night, with the help of Allen and a wheelchair, our family was reunited. Somehow, I felt everything would be OK.

The next day the geneticist spent hours with Henry. It appeared that everything else was fine. He was moved out of intensive care, and with the exception of the heart monitor and being on the small side, he looked like every other newborn in the nursery. The geneticist did some blood tests and we planned to meet with him a week later to review the results. That meeting went very well. Henry checked out fine. It appeared there was no connection between his small size, the thumb and the heart. There was one remaining test result to come in, but it was for a very rare genetic

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Adam

by Dottie and Rick Day

When Adam was diagnosed with Fanconi anemia three years ago, we learned that his pituitary gland did not secrete human growth hormone (HGH). At that time, growth was not the issue. Our main concern was to learn what we could do about FA. The fact that he would not grow to be six feet tall was the least of our worries. We had many conversations with our endocrinologist regarding HGH. He was a very compassionate, sensitive and intelligent man. He presented us with much data and statistics, and he offered guidance and referrals and made appointments with his mentor.

Rick and I listened. We heard of two FA-HGH patients in China developing leukemia. We did not know if they were already pre-leukemic before starting HGH, or if the HGH in fact caused the leukemia.

Unanswered questions—gather more data:

We attended symposiums and listened to professional opinion. Still, we could not make the deci-

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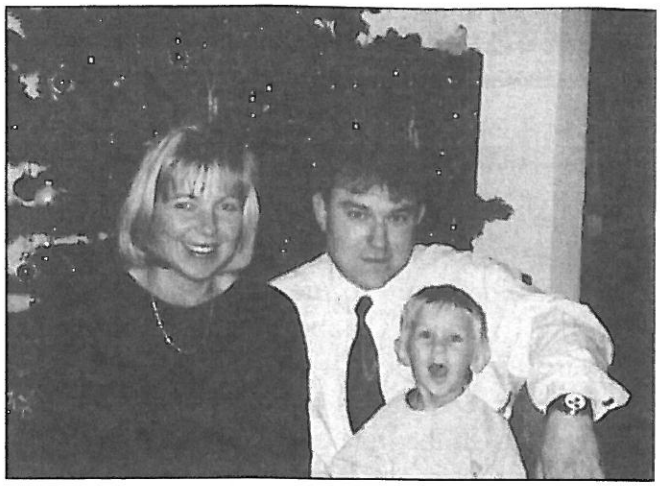
The Schmidt Family, Germany

by Michael and Claudia Schmidt, Braunschweig



Michael, Claudia, and Tim Schmidt

Tim is now almost 3 1/2 years old. When he was born, 3 weeks before his calculated date of birth, he weighed 5 pounds with a length of 46 cm (18 inches) and a head-diameter of 33.5 cm (13.2 inches). After his birth he was a small baby with a poor appetite and the following anomalies:

- broad nasal base
- right kidney too small and rotated
- reflux in left kidney
- micropenis
- café au lait as well as hypopigmented spots

Until the age of 2 he was prophylactically treated with antibiotics because of the reflux. Tim was a very restless baby; he never slept for longer than 3 hours. Still today, at the age of 3, he seldom goes to sleep alone and doesn't sleep all night through. During this time he is very restless. And our little sweetheart is a very bad eater. Some days we run after him the whole day with a spoon. On the other hand our Tim is almost non-stop in his activities; he is very vivid and charming.

By the age of 6 months Tim's growth had slowed down. Numerous tests showed that Tim's pituitary was defective. It did not produce any growth hormone, thyroid stimulating hormone or sexual hormone.

At the beginning of 1994 he started the growth hormone therapy, which meant one subcutaneous injection every evening. During this therapy his platelets declined continuously. He had petechiae (hemorrhages under the skin) at his thighs and lower legs. He was just 2 years old at this time. It was suspected that he had leukemia. A sample of his bone marrow was taken. The leukemia was not confirmed, but the chromosome break analysis in Heidelberg confirmed our other suspicion. Tim had FA! We asked ourselves again and again WHY. Why our Tim?

At this time Tim feels pretty well. We are happy and grateful and enjoy every single day, every single minute with him. Counts at this time (January '96) :

Hgb:	11.8
White:	7.3
Platelets:	78,000

Besides his growth hormone and the thyroid pills Tim gets no medicine at this time.

We try to nourish Tim very well and give him daily additional vitamins, and we fight for our child every day a little. We don't want to leave anything untried. We don't want to reproach ourselves with the fact that we have not taken all the chances, that we have not informed ourselves or others about FA. We enlist members for our German FA self-help group, we provide information about FA and raise money for FA research whenever we have the chance. We started our activities in September 1995, and from September to December we were able to raise about $10,000 for FA research—with the help of our friends and parents.

We would like to give special thanks to our friends Klaus Wegener and his wife for their great participation. We have raised money for research in the following ways :

* We wrote **charity letters** and received $500;
* We sold **cake and coffee** at the autumn fair of the neighboring town and earned $200;
* Prize money from a **card game tournament** was donated: $4,000;
* Children of our town organized a children's **Christmas play** and raised $900;
* During an **indoor cart race** $2,000 was collected;
* DEKRA (the Firm where Michael works) gave $3,000 at a **Christmas party**;
* For 1996 so far we have planned an **ice hockey** and **football benefit game**.

And last, but certainly not least, Tim's little brother Jan was born healthy (he does not have FA) on December 16th and made us very happy and grateful. ◆

Jake Siniawski and Sandy

Napoles Family at Camp Sunshine

by Virginia Napoles

I just don't have the words to express how happy we all were (Justine, Chelsea & I) to be at Camp Sunshine, Lake Sebago, Maine. It has been one of the most rewarding and nurturing experiences in our lives (especially for me).

I never imagined how compassionate, warm and lovable people can be, specially when many still have a very painful future ahead. The scientific presentations were very informative and important, since research brings hope and maybe a cure.

I wish I could just bottle up the whole experience, and when I am feeling down, bring it out and relive this wonderful experience we all shared. I still look around and think I will be seeing and hearing all the familiar faces we saw and heard for a week. The smiles, hugs, tears, small talks, kids laughing, parents relaxing and hoping for a brighter future. I find myself kind of lost, or not wanting to adjust to our daily routines.

Thank goodness I took a lot of pictures. Now we are able to take them out and again see all our new friends. We are looking forward to keeping in touch with some of the families.

Con mucho carino,
Virginia

The Siniawskis at Camp Sunshine

by Carol Siniawski

We have known about Jake's diagnosis for less than two years now, but still we chose not to attend the 1994 Family Gathering. I don't know, maybe we were afraid to see the future or maybe we were afraid to face so many other families just like us. They would know what we were going through and that somehow seemed a little scary. Maybe we were afraid that it would be more depressing than supporting. Anyway you slice it, we were afraid and did not go in 1994.

This year we felt stronger and were even a little excited to go. We only stayed the weekend, just in case it was not what we bargained for. I also decided to keep a journal and write in it every day.

Well, boy were we surprised! It was wonderful! We are sorry that we didn't stay the entire week and plan to stay all week next year. I am so glad I kept the journal, also. I was so surprised when I got home to find I had written several pages of thoughts, feelings, and observations. It is wonderful, now, to recount the activities that made the trip so worthwhile!

The accommodations were very nice. There was a very soothing environment right on our deck! The food was tasty and generous. Volunteers took great care of the kids. Jake had a great time making the pine cone bird feeder and making so many new friends. He may stay in contact with one of his new friends.

All three of those things made it easier to concentrate on why we were really there. The doctors' presentations were very interesting. My head hurt by the end of the day trying to soak in all I had heard, but the sessions were very helpful and enlightening. The question and answer sessions allowed us to ask more personal questions about our families in a much smaller group. It was great getting to know so many of the doctors who spend so much of their time on FA. Building those relationships alone makes a person feel better.

Most important were the family interactions. For instance, I have talked with many FA families on the phone, I have exchanged e-mail and US Postal Service mail with others, but I had only been in the presence of one other FA family. It was almost overwhelming to be in a room literally filled with people who know exactly what you are going through. It was amazing. Like I said, we have known a little less than two years and we found ourselves comforting, supporting and educating those who have known only six months, or one month, or one week. Others among us have known for five years and they are comforting and supporting and educating us. It was heart warming to have those kinds of discussions with each other. I can only hope that we helped a few families grow a little stronger, just as other families helped us grow a little stronger. The network you have worked so hard to create is strong and it is a wonderful thing. Thanks for your efforts, for your efforts will live long in the friendships we, the families, have formed. ◆

Amy Hayes

February 12, 1985 ~October 3, 1995

For four and a half years we have lived with the knowledge and the fear that this devastating disease could take our beautiful daughter, Amy, from us. And even though she had been getting progressively worse and more and more dependent on transfusions, we still couldn't believe that we would lose her. We always wanted to believe that by some miracle she would be spared the fatal prognosis of this disease. But on Oct. 3, 1995, our worst nightmare came true. Amy was only 10 1/2 years old and had just started fifth grade a month earlier, when she died of a brain hemorrhage.

Although we are thankful for the years and the cherished memories we have, our hearts will always cry out for more time. We are thankful that Amy was able to go to Disneyworld in August of '94. The USO and "Give Kids the World" organization provided us with a wonderful trip. We cherish the memories and pictures of how happy and excited Amy was.

One of the best things for Amy was when she got her central line placed in November '94. She was constantly being transfused with pRBC and platelets, and it was getting increasingly more difficult to find good veins. Not only did it make her many transfusions less stressful, but she was proud of the fact that she could draw her own blood and take care of it herself with the twice daily flushes. It helped her to feel that she had some control over a small aspect of these many medical procedures.

Amy grew up so fast and had to worry about things a child shouldn't have to worry about. This disease robs our children of the carefree days of childhood. Not a day (barely an hour) has gone by in the last four and a half years since her diagnosis that we have not thought about FA and what it was doing to our daughter. We know that every day for the rest of our lives we will think of Amy and the terrible disease that robbed her of the chance to grow up.

Even though our sons, Michael - 3 years and Brent - 5 months, tested negative for FA, we wish to continue being a part of the FA family. The newsletters over the years have helped tremendously. With each newsletter came the tears for the children and young adults who passed away. They also brought renewed hope for a cure or effective treatment being right around the corner. Although we wish we didn't have to share this common bond, it has helped to know that there are other families who know what we have gone through.

A very special, heartfelt "thank you" to Dave and Lynn for their time and dedication to helping the FA families and providing a network to keep families in touch and updated on medical news. God bless you for all you've given to the FA families.

We pray a cure can be found soon for this devastating disease.

God Bless,
Greg & Diane Hayes

We Welcome New Families Who Have Joined Our Support Group

Janeth & Al Acosta
80 Van Hauton Avenue
Chatham, NJ 07928
(201) 701-1094
Michael - DOB 8/7/91

Linda Bledsoe
14908 Ritter #4
Victorville, CA 92394
(619) 955-7784
Timothy - DOB 8/6/90

Marsha Brock
2713 Walnut Street
Blue Island, IL 60406
(708) 385-9079
Caryl - DOB 6/3/82

Melinda Burgos
5 Cedar St., lst Floor
Haverhill, MA 01830
(508) 373-1980
Melissa - DOB 2/11/81

Mike & Bronwen Carr
Millstones Kings
Lane Barrowden
Ruckland LE158EF
United Kingdom
(011)1572 747-861
Toby - DOB 9/19/81
Marcus - DOB 5/5/79

Diane Edmundson
24320 Silver Bullet Way
Murrieta, CA 92562
(909) 677-4632
Christine - 8 years old

Amber Garthus
PO Box 174
Warroad, MN 56763
(218) 386-3386
27 years old

Darla Lindenmayer
1005 Webster Court
Jeffersonville, IN 47130
(812) 283-7122
Matthew - DOB 7/3/90

Lisa & Jack Nash
10191 E. Crestridge Lane
Englewood, CO 80111
(303) 773-6228
Molly - DOB 7/4/94

Laurie Strongin & Allen Goldberg
3839 Calvert St., NW
Washington, DC 20007
(202) 342-2710
Henry - DOB 10/25/95

In Loving Memory

Amy Hayes
2/12/85~10/5/95
Andrew Hilaire
3/5/85~12/26/95
Kavin Hume
5/1/60~11/24/95
Timothy King
11/13/87~11/6/95
Byron Poe
8/18/85~11/9/94
Adam Ingvaldson
11/20/87~1/20/95

Changes of Address

Donna Barnes
804 E. Lackawanna
Olyphant, PA 18447

Courtney Hilaire
11164 Barbizon Circle E.
Jacksonville, FL 32257-7082

Wayne & Carol Ingvaldson
3953 Goodwin Ave.
Burnaby, BC V5G 4A1
CANADA
(604) 473-5091

Les & Barb Lawrence
Box 3-C Comp 2
Tulameen, BC V0X 2L0
CANADA
(604) 295-6230

Karl & Vanessa Meyer
PO Box 408
De Deur
South Africa 1884

Mike & Pam McCoury
1009-B, Box #6
Timesville Road
Signal Mountain, TN 37377
(423) 886-9678

Joanne Sileo
405 W. Centre
Woodbury, NJ 08096
(609) 845-5961

Sheila Zanutto
5583 N. Franklin Blvd.
Merced, CA 95348
(209)384-5895

Adam Day
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sion. It was constantly in the back of our minds, uncomfortably, tucked away back there. Adam had had a rough winter. His ANC had begun to be a problem. By this time he was enrolled in the G-CSF study at Riley's Hospital for Children in Indiana. He was receiving two injections daily. Adam did not mince words when expressing his discomfort. In the months to follow, his ANC did pick up, and he was still six years old and thirty-seven inches tall. One cold day, Adam came home from school shattered. It wasn't the first time that he had been called "shorty" or taunted because of his size, but apparently he had heard all that he wanted to hear. He cried and cried and said he didn't want to be little anymore.

Rick and I discussed Adam's happiness and quality of life. There was no question in our decision. After conversing with experts in this field, Dr. Shahidi's words continued to echo in our minds, "If the child is definitely growth hormone-deficient I urge you to begin treatment." We talked with Adam about receiving another injection. By this time his G-CSF injections had been reduced to once a day. We assured him that the HGH would help him to grow taller. With all three of us in agreement, we began HGH on April 1, 1994. Adam was thirty-seven inches tall and weighed approximately 30 lbs. Today is December 12, 1995. Adam is forty-eight inches tall and weighs 65 lbs.

During this time, Adam's hemoglobin had fallen, so he also began androgen therapy in August 1994. At this point his dose of HGH was increased to the maximum for his weight, in order to produce maximum growth before his growth plates closed from the androgens.

Was putting Adam on HGH the right decision? We three believe it was, at least for us. There may be no right or wrong decision on this issue. We know that seeing Adam as tall as his peers has given him a small corner of normalcy in this world. We believe that it's a personal decision based on your feelings and physician's guidance. Would we do it over? Under the same circumstances—yes. If anyone would like to speak with either Rick or me regarding this issue, please feel free to contact us. We are both in the directory. ◆

Comments from 1995 Camp Sunshine Participants

- Being a single parent, I was apprehensive—would I have enough help with the kids? Rest assured all was so well planned and the volunteers so kind & willing. I never once worried about my kids! It's the perfect way to allow your child a "NORMAL" society. They, as well as us, can be in a (nobody different) world of normalcy.
- Children enjoyed the activities immensely. The volunteers did an excellent job! It's the ideal family vacation spot! The boys did not want to leave. We got to become acquainted with more families at Camp Sunshine than at the previous family meetings. This is very important as we all need the support.
- This is my first year. I am really impressed by the quality of this camp. It is awfully excellent.
- Evening campfires, shared episodes. Love with people I just met.
- We loved it. The love, support and care that was felt throughout the week. Everyone watching over everybody's children. The hugging and smiles shared by almost everyone. The vast knowledge acquired from all the speakers and scientific presentations.
- My son says Camp Sunshine is the only good thing that ever happened to him because of FA. We look forward to this all year.
- I enjoyed the time close with my child & leave here with hope!
- It is so wonderful. I looked forward to MAY, 1995 ALL YEAR LONG. Ask anyone!
- What can I say? The place is perfect!
- I will come back next year.
- Pat & I both thought that this would be our last time here, but we'll be back.
- The experience my wife & I had here at Camp Sunshine was outstanding. From lodging to the volunteers. The effort was 1st class and the results were second to none.
- Camp Sunshine is WONDERFUL.

FUNDRAISING

FA Families Surpass the Tiger Challenge in Record Time

FA families and friends sailed past their $50,000 challenge from the Tiger Foundation of New York in early December, 1995 and raised $86,181 by year's end.

Many thanks to the Tiger Foundation for its generous support. Congratulations to all who have worked energetically at all levels to support FA research over the past year.

We must continue our efforts in equal measure in 1996. As this newsletter reveals, FA research is moving ahead rapidly. With your continued support, the FA Research Fund Board and Scientific Advisors will direct every available dollar toward understanding, treatment, and a cure for FA. ◆

We Honor Our Benefactors January 1 – December 31, 1995

Amgen Corporation
Andersen Construction Co.
Donald E. Barker Foundation
Blue Cross/Blue Shield of Oregon
Phyllis Cafaro
Collins Medical Trust
Graham Covington and Graham's Crackers
Fluhrer Properties
Georgia Pacific Corporation
Larry and Anna Gould
Samuel S. Johnson Foundation
Klarquist, Sparkman, Campbell, Leigh & Whinston, Attorneys
Phil Knight
Louisiana Pacific Foundation
Meyer Memorial Trust
Nike Matching Gift Program
Oregon Community Foundation:
Edwin and June Cone Fund
The Bowerman Fund
The Summerville Fund
R. B. Pamplin Corporation
The Pape Group, Inc.
Sandoz Foundation
F. E. Stewart
Patricia and William Smullin Foundation
The Swig Foundation
Taylor Winfield Foundation
Tiger Foundation
Timber Products Co.
Trinity United Methodist Church, McMurray, PA
Rose E. Tucker Charitable Trust

We also send our deepest appreciation to the many thousands of caring families, friends, and scientists who contribute to our mission around the world.

Boston FA Research Fundraiser Huge Success!

by Mike Vangel

On October 28, 1995 a Halloween costume party was held to benefit FA research. The party was the highlight of a fundraising drive that included writing donation letters to family & close friends and selling raffle tickets for great prizes. We rented the National Guard Armory in our hometown of Hingham (a small but quaint coastal community just south of Boston). The armory was a perfect choice for three reasons. First, we got it for free. Second, it was large enough to accommodate up to several hundred people comfortably. Third, it was constructed over 100 years ago and at nighttime looked like a haunted castle (with a little help).

Beth and I did not attempt this alone. We had a core group of about a half dozen friends and family who helped tremendously. None of us had ever attempted anything like this before but that did not get in the way of us making it happen. While we are not financially well off, Beth and I are rich in what really counts: a strong and close-knit family, good friends and a supportive community.

The party was held in the evening from 8 pm-1 am and a great time was had by all. Prizes were awarded for best costumes, and the raffle winners were announced. Over 300 people attended the event, most in cos-

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Boston Fundraiser

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tume! We charged $15 per person to attend and we provided plenty of food and a DJ for entertainment. Grand Prizes included a baseball autographed by Jose Canseco (provided by Ed and Jan Duffy), a hockey stick autographed by the Boston Bruins (also provided by the Duffys), a baseball autographed by Cal Ripken Jr., weekend getaways at the Boston Harbor Hotel, Sheraton Boston Hotel, and Kimballs by the Sea resort. Other grand prizes included dinner cruises aboard the Spirit of Boston and the Odyssey, vacation rentals at homes in New Hampshire and Cape Cod, and many restaurant gift certificates. We raffled over 100 prizes altogether! And I know we passed the litmus test for a good party when the police were called to shut our event down at 1 am!

Altogether we raised over $23,000, including the revenue generated by the party, raffle ticket sales, and donation letters. Surprisingly, more than half that amount was earned through raffle ticket sales. And believe it or not, checks are still streaming in! Was the $23,000 earned overnight? No, it took a lot of planning and effort but it reaped more than cash rewards. For both Beth and me, it was a healing experience to come to terms with our daughter Amy's diagnosis and to do something positive about it. Also, since Amy was diagnosed only last June, the fundraiser provided us with a way to reconnect with family and friends. Odd though it may seem, after the diagnosis, rather than reaching out for support, we withdrew. The whole situation seemed so unreal and we felt so isolated. For us, it was painful to talk to anyone. Organizing the fundraiser provided us with a way to confront our situation, meet other FA families like the Duffys and Curtises, and do something positive to help Amy and all other FA children to move forward with their lives.

> "For both Beth and me, it was a healing experience to come to terms with our daughter Amy's diagnosis and to do something positive about it."

Note: If you would like to put together a fundraiser but don't know where to begin, please get in touch with us. I've put together some common sense guidelines and may be able to save you time and aggravation. Remember, if we could do it, you can. We didn't set out to raise $23,000. Even if we had raised only $2,300 it would have been a success! You can make a difference!! Special thanks to Debby Slater who inspired us!

Mike & Beth Vangel
15 Free Street
Hingham, MA 02043
(617) 740-2034

Your FA Research Dollars at Work
January 1 - December 31, 1995

Complementation Group Studies and Gene Identification	
Hans Joenje	$ 64,015
Fré Arwert and Chris Mathew	169,762
Arleen Auerbach	43,684
Understanding the Function of the FA Protein	
Maureen Hoatlin	58,400
Development of Effective Treatments	
Stem Cell Bank Project	50,000
Total Funded	$398,961

Henry Strongin Goldberg

continued from page 7

illness, and we were told we had no reason to worry.

Only all of you can know the devastation Allen and I experienced at the news that Henry has Fanconi anemia. We are in the process of learning all we can about this disease and what we can do to help Henry. The people involved in this network have provided information without which we would be lost. Although we wish more than anything we had never heard of FA, now that we have, we plan to do everything in our power to give Henry all the love and care we have to offer.

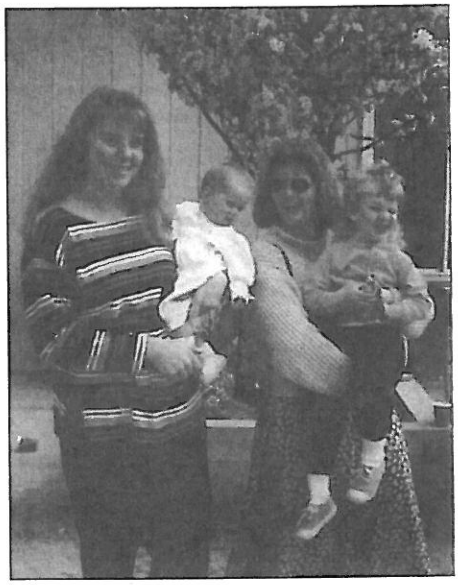
Our son is a strong person with a wonderful spirit. At 10 weeks, he has exceeded all predictions about the timing of his first heart surgery. Although we know it will come soon, I don't yet know how we will be able to hand our beloved son to the doctors on that dreaded day or how we will make it through the excruciating wait during the procedure. What I do know is that without the treatment he will not get better. And with this surgery and those to follow, Henry will be that much closer to being a healthy, strong boy.

I am currently searching for the strength within me to deal with all of this. I know it is there and that Allen, Henry and I, together with all of our wonderful and loving family and friends, and with the support of this network will thrive as a family. ◆

FA Web Page

Jeff Janock, a member of our FA Family Support Group, is constructing an FA Web page. He has links to several interesting sites, including the HLA Registry Foundation, Inc., with information on how to become a bone-marrow donor. Jeff is also planning to publish our Handbook on his Web page.

http://bc.cybernex.net/~jj/fa



L-R: Lori and Emily Salo and Donna and Thomas Barnes enjoy a break in the action at Camp Sunshine

Camp Sunshine

continued from front page

on Friday morning, May 17. Families will be treated that evening to the wizardry of professional magician Bruce Johnson. Mr. Johnson's appearance will be sponsored by Ron and Fredi Norris in honor of their son, Alex. Each day and evening that follows will be packed with new knowledge and friendship and ultimately, as Camp Sunshine veterans report, new strength.

Check the program outline and mark your Early Registration card, and make your travel plans. ◆

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