

# THE FA FAMILY NEWSLETTER

Newsletter Number 7

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## FA SCIENTIFIC WORKSHOP BRINGS REPORTS OF PROGRESS

Fifteen leading research scientists and expert treating physicians met in Portland, Oregon on October 28th and 29th, 1989 to review work in progress on Fanconi Anemia. This intensive workshop was sponsored by the Fanconi Anemia Research Fund, Inc. and funded by the generous support of the Oregon-based Chiles and Bowerman Foundations, Louisiana-Pacific Corp. and a gift from Michael Greenberg, M.D.

The workshop assembled key FA researchers from the United States, Canada and Europe. From the reactions of participants and our Board's observers, the meeting was highly successful. It clearly has speeded progress on a variety of fronts.

The Fund held the workshop (1) to hear progress reports concerning funded projects; (2) to assess progress of research into gene discovery; (3) to identify areas for potential future research funding; (4) to stimulate research interest in developing therapies and an ultimate cure for FA; and (5) to encourage increased collaboration among research projects. All of these objectives were advanced.

The following report is somewhat technical, but we report at length to give families and their treating physicians a flavor of the exciting developments. Please bear in mind the usual disclaimers that your editors are not scientists. These are lay summaries for lay readers.

### Genetic Research

Three laboratories reported progress in

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pursuit of three different strategies to isolate the FA gene or genes. Each will ultimately complement and test the validity of results from the others.

1. Dr. Arleen Auerbach of The Rockefeller University is using RFLP markers in a "linkage analysis" study of FA. Her approach has been described in previous newsletters. Dr. Auerbach's laboratory has now completed probes that cover approximately 30% of the human genome.

2. Dr. Robb Moses of the Baylor College of Medicine is pursuing a "cDNA library transfer study" to attempt to clone and recover a gene which corrects the cellular defect in an FA cell line.

3. Dr. Margaret Zdzienicka of Leiden, The Netherlands reports attempts through gene transfer technology to clone a gene in a Chinese hamster mutant cell line that is equivalent to FA. Her laboratory and its international collaborators are highly respected.

In addition to these approaches, Dr. James Boyd, whose research is supported by our Fund, reports that his Davis, California lab "has recently identified a new enzyme property of tissue culture cells derived from FA-A (\*) patients. Preliminary studies suggest that this may permit a more efficient means to distinguish between group A and non-group A disorders. Further characterization of this property is expected to lead to an improved understanding of the molecular defect associated with the group A disorder. That research group has also identified a gene in the model genetic organism *Drosophila* (fruit fly) that shares many properties with the FA-A gene. Attempts to clone that gene can potentially lead to the recovery of DNA sequences that can in turn be used to clone the corresponding human gene."

Dr. Moses summarizes the significance of these strategies as follows:

"Fanconi anemia research has surged ahead at the molecular level. Several independent efforts by different laboratories suggest that the isolation of the FA gene (or genes) and localization on the human genetic map are real possibilities. While much work must still be done, evidence presented at the meeting indicates that the technologies are available to accomplish those feats.

What will this mean to patients and parents? Hopefully a great deal, but one must be conservative in outlook. The gene will allow unambiguous identification of affected individuals and carriers (not yet available). Moreover, it should point the direction for research on treatment. This will allow efforts to be focused in the correct area and enhances the chance of developing a successful therapy."

\*Editors' note: "FA-A" refers to complementation group "A" that characterizes some but apparently not all FA victims. The existence and significance of multiple complementation groups in FA is poorly understood and was much discussed in the FA Portland workshop. Participants in the workshop were universally respectful of the pioneering work in this area of fellow participant Manual Buchwald, PhD of the Hospital for Sick Children in Toronto, Canada.

## **Therapies**

Research has demonstrated that androgen therapy is life-extending for many FA victims. Marrow transplantation is a potential cure for those fortunate families with HLA matching donors.

Colin Sieff, M.D. of the Dana-Farber Cancer Research Center, Boston, Mass.

discussed new exciting therapeutic approaches to FA at the October workshop in Portland. We quote and paraphrase from his written summary following the workshop. This report is both speculative and hopeful. It is technical. Please review any questions with your treating physician or medical consultants.

"To turn to therapy, several exciting prospects were discussed....Possibilities for hematopoietic growth factor (HGF) therapy include granulocyte-macrophage colony stimulating activity (GM-CSF) and interleukin-3 (IL-3), most active on primitive stem and progenitor cells as well as mature cells; and erythropoietin (ep) and granulocyte CSF (G-CSF). The latter are active on more mature progenitors.

Our evidence from serum-deprived cultures suggests that combinations of growth factors are necessary for full in vitro colony maturation. For example, GM-CSF or IL-3 plus ep is required for erythroid colonies. Further, the combination of GM-CSF and IL-3 is additive or synergistic on all cell lineages (erythroid, granulocytic, and monocytic). This in vitro evidence has been corroborated by in vivo simian (monkey) studies. Monkeys given IL-3 followed by low dose GM-CSF show a much greater response (in erythroid, myeloid and platelet lineages) than animals given either factor alone. Human studies are not as advanced.

We have treated 9 children with moderate to severe acquired aplastic anemia with GM-CSF alone, and results are evaluable in 8 patients. Bone marrow cellularity was less than 5% in seven out of eight patients and less than ten percent in one out of eight patients. Seven out of eight patients completed the 4 week course; six of seven responded with a 3-fold increase of median PMN count to 1200/cu mm. All six had PMN of greater than 1000/cu mm by day 28. Four of six have been treated

for more than 12 weeks. Two of three rebiopsied have increased bone marrow cellularity to 40-60%. One patient had a trilineage response that has been sustained for a year.

"...These results are encouraging, and suggest that GM-CSF may be palliative by increasing granulocytes to protective levels. Side effects are usually minimal. Current planned trials at The Children's Hospital include:

1. Recombinant GM-CSF in pediatric patients with FA.
2. Recombinant IL-3 alone, then followed by GM-CSF in patients with severe acquired aplastic anemia.
3. Recombinant IL-3/GM-CSF in patients with FA.

"...For patients who lack bone marrow donors and are unresponsive to androgens, HGF therapy might offer a very effective means of managing bone marrow failure. If it is not unduly toxic, it could replace or complement androgens as the optimal maintenance therapy."

## **FA RESEARCH SUPPORTED BY TWO AFFILIATED GROUPS**

We are pleased to report that two additional family support and scientific research fund-raising efforts are underway. We have been in touch with these efforts and met their founders. Our mutual work is supportive, not competitive, and we welcome these projects and wish them well.

Michael Greenberg, M.D. of Philadelphia, Pennsylvania is in the process of forming an

FA research fund under the laws of his state. 501 (c)(3) status is expected, and we shall report all details when they become available. Dr. Greenberg attended and helped support the October workshop.

Dr. Giovanni Pagano, who also attended the October symposium in Portland, has formed the "Associazione Italiana per la Ricerca sull'Anemia di Fanconi", as of June 1, 1989 in Naples, Italy. Dr. Pagano has assembled a number of Italian FA families and is helping conduct fundamental research in his country. A modest grant from FA Research Fund, Inc. helped with start-up costs for his research project.

## **RESEARCH IN PROGRESS: A CONCLUSION AND A CALL FOR CONTINUED ACTION!!**

Your editors left our first workshop with a multitude of hopes. First and foremost among them is the universal conclusion that progress in finding the gene or genes that cripple our mutual family futures IS A MUST.

We are organized; we now have scientific support at the most determined levels. But research funds are indispensable. Consider the following comment on results of your fundraising efforts by a leading scientific researcher who wrote to us following the conference. It shows that you - and we - already have made a difference:

"(F)or a small volunteer organization with no experience you performed a near miracle. For those who labor in the trenches I can't begin to tell you how grateful I am for your warm personal introduction to the FA Community you have built....Probably the

most important contribution of such a gathering is the establishment of personal contacts....In this regard I was able to extend current collaborations with two laboratories and to initiate two new collaborations."

**YOUR FUNDRAISING EFFORTS ARE VITAL.  
REDOUBLE THEM! MANY FAMILIES HAVE  
MADE EFFORTS, BUT MANY MORE MUST.**

## **TRANSPLANT DEVELOPMENTS: Marrow of FA Victims Rebuilt With Umbilical-Cord Blood From Newborn Siblings**

Two FA patients now have received life-saving therapy from a new source of "stem cells" that reconstitute bone marrow. This procedure, conducted by French and American collaborators was reported in the October 26, 1989 issue of the New England Journal of Medicine and has received substantial media attention.

Our newsletter advisor, Arleen Auerbach, PhD of The Rockefeller University is a principal author of this exciting report. The parents of a boy with FA discovered through pre-natal tests that an unborn and non-FA affected child was an HLA match for their FA son. At birth the umbilical cord blood of the newborn sister was painlessly drawn, frozen and stored for seven months.

Researchers have long known that umbilical cord blood is rich in the precursor "stem cells" that are the source of all later stages of blood system development. The transplant from cord blood was delayed only to insure that the newborn daughter



might be a "back-up" source of bone marrow if the cord blood was unsuccessful.

Dr. E. Gluckman in Paris, France conducted the transplant in September, 1988. After drugs and radiation were used to destroy the defective FA marrow, the cord blood successfully replenished the marrow of the FA victim. He now "leads a normal life", according to a research report in Science News, November 4, 1989.

On September 14, 1989, a second successful cord blood transplant for an FA child occurred in Paris, France. Natalie Curry, age 4 received the transplant from her sister Emily's cord blood. Complications have been minimal (she had a small rash on the palms of her hands indicating very mild graft vs. host disease) and engraftment of the new marrow from Emily's cord blood was unbelievably rapid. Natalie was able to leave the hospital on October 10th, and returned to the United States on November 17th. Her counts are now normal, and if all continues to progress as expected she should be able to resume all normal activity within six months from her transplant.

Dr. Auerbach suggests several advantages of this new approach over marrow transplants. Sometimes painful marrow extraction from matching siblings is avoided. The sick child can receive a transplant immediately after the birth of a healthy matching sibling, rather than waiting for the donor child to reach the minimal donation age of six months. Other researchers have suggested that using immature cells might make the serious complication of graft vs. host disease less likely.

These cord blood transplants raise important questions. Could this method

be successful if the donor were not a perfect HLA tissue match? Can one extract enough cord blood to reconstitute the bone marrow of an adult-sized recipient? According to Dr. John Hansen of the Fred Hutchinson Cancer Research Institute, answers to these questions will probably not be forthcoming until additional transplants are attempted. For the moment we delight in the success of the two transplants successfully completed to date, and anticipate that other FA children will benefit from this new, important advance.

We are aware of serious and important ethical and medical issues surrounding this procedure. This is a matter for discussion with principal researchers and your own treating physician.

## **FA RESEARCH FUND FORMED; MERGED WITH FA FAMILY NEWSLETTER**

As an integral part of the fundraising efforts described in newsletter No. 6, the Fanconi Anemia Research Fund, Inc. was formed early in 1989. The Fund is a non-profit Oregon Corporation. The Fund has received 501 (c)(3) status from the Internal Revenue Service, effective as of February 27, 1989. As a consequence, gifts to the Fund are tax deductible to the donor. The operations of the FA Family Newsletter have been transferred to the Fund, so that donations to the newsletter likewise are tax deductible.

Potential major donors may need the IRS employer identification number of the Fund. It is 93-0995453. Any questions should be directed to Dennis Solin, CPA, President of the Fanconi Anemia Research Fund, Inc., 66 Club Road, Suite 300, Eugene, OR 97401. Phone (503) 344-6307; FAX (503) 484-0892.



## THE ROCKEFELLER UNIVERSITY

1230 YORK AVENUE • NEW YORK, NEW YORK 10021-6399

November 27, 1989

Dear FA Families:

As you have read elsewhere in the newsletter, a superb workshop on Fanconi anemia research was held in October, in Portland, OR. All of us working in this field had an opportunity to hear the latest reports of what others were doing, and to interact regarding suggestions for future research.

We are intensively proceeding with our linkage analysis studies to locate the Fanconi anemia gene. We have completed analyzing data from DNA probes covering approximately 30% of the genome. These studies will continue until we have mapped one or more genes for FA. We are especially grateful to one of our FA parents, Dr. Michael Greenberg, who recently gave us a donation to purchase a special computer that will help expedite these studies.

I will be leaving tomorrow to attend hematology meetings in Atlanta, GA, where I will present some new data from the Fanconi Anemia Registry, dealing with prognostic factors for survival. We now have 222 non-European families in the FA Registry. The median estimated survival for these patients is 25.17 years. The proportion of patients still alive at age 10 is 80% and at age 20 is 58%. The median age of onset of hematologic problems is 6.24 years. At 10 years of age 25% of patients have not manifested hematologic problems. Estimated survival for those patients with onset of hematologic problems later than 6.24 years is especially good. Lack of serious birth defects such as gastrointestinal or kidney malformations also correlated with later onset or better survival. These data will be written up in more detail for publication. Please note that these data are obtained by a statistical analysis of information from a large number of patients, but that there is a great deal of variation in the clinical picture of FA. Each patient is unique, and we can't predict for certain the course of the disease in an individual patient.

We appreciate hearing from parents so that we can update information on your child for the FA Registry. We have a new coordinator for the Registry starting December 1st, who has a degree in genetic counselling. When you call (212-570-8862), please ask to speak to Ms. Barbara Adler.

My best wishes for the upcoming holidays.

Sincerely yours,

Arleen D. Auerbach, Ph.D.

We report with deep sadness the passing of nine year old John MacLellan of Brampton Ontario, Canada. John died following a brain hemorrhage in the spring of 1989, and will be greatly missed.

His classmates at Bishop Francis Allen School wrote moving tributes to this generous, patient, kind, humorous friend who "never felt sorry for himself". John's mother, Moira, wrote a touching remembrance from which we quote in part:

" 'Don't worry about it, Mum!' It seems that these were the words that John used to me most often those last few weeks. He didn't want any fuss, he just wanted to be 'normal', for want of a better word. We learned a lot from him. He accepted people as they were.

A friend of mine, Joanne, was a developmentally delayed person. She became his closest friend, and he told her that 'he wouldn't be sticking around for too much longer'. He admired Joanne's honesty, and just liked her, no matter how she looked, etc. Even as a little fellow in nursery school, I remember his teacher saying how he was the only one who had patience with Mickey, who was deaf and dumb, and often got frustrated, and threw things. He was very concerned with social injustices, and had a great sense of fair play. He had a great sense of humor, and loved to tell jokes, despite the fact that he was shy and reserved.

We miss him so much, and wish it could have been different. Still his death has not been in vain, in that now friends realize just how terrible Fanconi's is, and they all want to help. None of us ever really expected John to die, and he lives on in our memories."

## **NUTRITIONAL SUGGESTIONS FROM ITALY**

Giovanni Pagano writes from Naples, Italy of his decision to change the dietary habits of his family to minimize carcinogenic (cancer-causing) risks to his FA son. Dr. Pagano's review of nutritional literature led him to suggest that FA patients minimize intake of excessively broiled or deep-fried food, excess fat and "quite obviously, alcohol". Dr. Pagano also recommends intake of fresh fruits and fruit juices, green leafed vegetables, vegetables such as broccoli, cauliflower, cabbage and brussell

sprouts, garlic (preferably raw) and fish and fowl rather than red meats. He also has chosen to avoid foods which have been grown with food additives, pesticides and antibiotics.

As Dr. Pagano notes, "It should be clear that diet habits are not a 'new hope' for our common problem. Nevertheless, we should be aware that some wrong diet habits could be an additional risk factor for a weak organism that is very susceptible to environmental factors....Diet habits should be at least a matter of prudence among FA families."

Jackie and Bill Lucarell of Girard, Ohio wrote this lovely letter to Lynn and Dave Frohnmayer and share it with all FA families. We are deeply inspired by the efforts of this family.

"Thank you for enabling us to fight back by raising funds for FA. Our fundraisers have been overwhelming successes due to the untiring efforts of some very special people.

It is imperative that the news media know about us and what our goals are. It was you, Lynn, who inspired me to go to our local news media. We had nothing to lose and everything to gain. We resolved that we would not let Jimmy go downhill without trying to save his life and the lives of other children afflicted with FA.

In early January, after your presentation on USA Today, I decided to go to our local newspaper, The Vindicator and to a local TV station, WFMJ. We're not publicity hungry, but face it, none of us can afford to be apathetic. We desperately need research for this killer disease! I gave them our story about Jimmy and his battle with FA. The results were well worth it. We never could have raised over \$25,000 for FA if we had not generated news media interest.

Everything started to happen almost spontaneously. We found out that people are pretty wonderful and are more than willing to help. All that we did was let them know about our dilemma and our urgent need for funds for FA research.

Jimmy's preschool, Great Beginnings, launched a very successful candy drive along with two bake sales organized by some very dedicated individuals. Our church, St. Patrick's - Youngstown presented a wonderful spaghetti-chicken dinner with all the proceeds going to FA. The school children of St. Patrick's raised \$350 by having pizza sales and by doing very innovative projects at school in order to help Jimmy.

And last month our Girard beauty salons and a terrific committee organized a Cut-A-Thon accompanied by a bake sale and a craft sale. They had a great turnout and raised \$2200 for FA!

Due to the publicity in the newspaper we have received (and continue to receive) numerous monetary donations to the FA Research Fund.

Our wonderful families have been instrumental in soliciting funds for FA by asking their friends, relatives and business people to contribute. People do care! We have to let them know we're out there! Our mission is saving the lives of our children and loved ones. We can't afford to be complacent. If we don't take the initiative, who will? Together we can and will make a difference!

Without our faith in God, the support of our family, friends and benefactors, and the leadership of Dave and Lynn, we could not have made it through these difficult times.

Jimmy, age 4 1/2 was diagnosed as having FA on December 6, 1988. Thank God he is enjoying a period of stability, which we hope lasts a long time. In the meantime, we just keep praying for the strength to fight back. Our prayers are being answered. We have that strength and pray that all our FA families will have it, too!

Love, Jackie & Bill Lucarell "





The Vindicator

**Awaiting a cure:** William and Jacqueline Lucarell of Girard, shown here with their children, Billy, 7, left, and Jimmy, 4, seek funds for ongoing research into a cure for the rare hereditary Fanconi's

anemia which Jimmy has. Fewer than 200 people in the country are afflicted, and most victims die before reaching adulthood. The illness limits production of platelets and red and white blood cells.

# 4-year-old fights rare disease

## Illness cuts output of blood cells

By Peter H. Milliken

Vindicator staff writer

GIRARD — A 4-year-old boy living here is one of fewer than 200 persons in the nation known to be afflicted with a rare hereditary form of anemia whose victims usually die before reaching adulthood.

Jimmy Lucarell, the son of William and Jacqueline Lucarell, of 2 Mohawk Drive, has Fanconi's anemia, a progressive illness in which a missing gene limits the body's production of platelets and red and white blood cells.

One in 300 persons is a Fanconi's carrier. For a child to have the disease, both parents must be carriers. Then, the chances are one in four that a child will have the illness. No test exists for carriers.

The only real remedies are to find a cure for the disease or a perfectly matched bone marrow transplant.

Research on the cure, which may be found about five years from now, is being conducted by Dr. Ariene Auerbach at Rockefeller University in New York City.

The goal of the research is to find and clone the missing gene

and insert that gene into the victims' bone marrow.

Since none of Jimmy's relatives have been found to have a human leukocyte antigen (HLA) type that matches his, an unrelated bone marrow donor would have to be sought if Jimmy is to go the transplant route.

Jimmy is on the national registry as a potential bone marrow recipient, but the chances of finding an unrelated donor to match him are about one in 20,000.

**'WE'RE putting our trust in God. Ultimately, it's His will. We're putting in a lot of prayers that they can find a cure for Fanconi's anemia.'**

*— Jacqueline Lucarell, Jimmy's mother*

Some 425 Americans now need bone marrow transplants.

Because non-related bone marrow transplantation carries with it a high risk of rejection and death for the recipient, it would be a last resort for Jimmy, probably years from now, said Dr. Mustafa Barudi, the pediatric hematologist and oncologist from Tod Children's Hospital who diagnosed Jimmy's condition.

"We're putting our trust in God. Ultimately, it's His will. We're putting in a lot of prayers that they can find a cure for Fanconi's anemia," Mrs. Lucarell said.

On Jimmy's third birthday, Mrs. Lucarell requested some blood

tests because Jimmy seemed pale and thin.

The family pediatrician initially diagnosed the ailment as iron deficiency anemia.

This past fall, Mrs. Lucarell noticed Jimmy was bruising and suspected leukemia.

"He was picking up infections more frequently than other children, and he couldn't seem to get rid of them," Mrs. Lucarell said, adding that she asked the pediatrician to repeat the blood tests.

The tests showed low platelet and hemoglobin levels, and the pediatrician referred Jimmy to Dr. Barudi.

At first, Dr. Barudi noted Jimmy's thumb malformations as a Fanconi's symptom. He then diagnosed Fanconi's based on a bone marrow biopsy and chromosome test.

The diagnosis was confirmed by Dr. Susan Shurin, a pediatric hematologist at Rainbow Babies and Children's Hospital in Cleveland.

In December 1988, Dr. Barudi prescribed androgens for Jimmy to stimulate the bone marrow to produce platelets and red and white blood cells.

Jimmy has so far had one blood transfusion and five hospitalizations in the past three months, once with a fever of more than 105 degrees.

Because an infection might threaten his life, Mrs. Lucarell has had to withdraw Jimmy from the Great Beginnings Preschool in Girard until his white blood cell count rises to a safer level.

Jimmy's 7-year-old brother, Billy, who attends Washington Primary School in Girard, is now undergoing tests for Fanconi's anemia, and results are still awaited.

Jimmy's father, an electrical engineer at Taylor-Winfield in Warren, said the family's top priority now is to raise money for research into the cure for Fanconi's.

Donations may be sent to the FA Research Fund, Box 477, Girard, Ohio, 44420.

Those wishing more information about potentially becoming bone marrow donors may contact Carolyn Kean, bone marrow donor coordinator, American Red Cross, 3950 Chester Ave., Cleveland 44141.

"The people of Girard, our friends and neighbors have come forward with prayers and gifts and food and genuine concern and we're deeply touched by all of that. Our family has been very supportive too," Mrs. Lucarell said.

Mrs. Lucarell concluded, "Our priorities are our faith and our family. Our lives have centered around our children. They always did and they always will."

## **SCIENTIFIC ADVISORY COMMITTEE FORMED**

We are pleased to announce that Grover Bagby, M.D., Professor of Medicine and Medical Genetics at the Oregon Health Sciences University serves as head of the scientific advisory committee for the Fund. Dr. Bagby has been an NIH researcher and reviewer and has served as an editor of leading journals in hematology and oncology. Dr. Bagby has assembled a panel of eminent hematologists and molecular biologists to assist in evaluating proposals.

Requests for funding through the Fanconi Anemia Research Fund, Inc. are sent immediately to Dr. Bagby and his colleagues. The Board of Directors of Fanconi Anemia Research Fund, Inc. relies heavily upon the expert scientific and medical opinion of the scientific advisory committee in deciding which research proposals to support. The assistance of Dr. Bagby and his professional colleagues assures that only projects of high and reputable scientific merit will be supported by the Fund.

## **LINDA SOLIN COORDINATES ACTIVITIES OF FA RESEARCH FUND, INC.**

Linda Solin, a student in the University/Community Action program at the University of Oregon, is serving a nine month field placement as coordinator for our Fund. Linda did a superb job of organizing the October 28-29 workshop on Fanconi anemia. She has applied for grants for our Fund, is involved in all aspects of fundraising, and is responsive to many needs of our

families. Her tireless work as an intern with our project has helped us all make great strides in promoting vital research and family support. Many thanks, Linda.

## **UNRELATED MARROW DONOR PROGRAM VOLUNTEERS; CONGRATULATIONS TO LYNNETTE CHANDLER**

Lynnette Chandler's daughter Amanda, age 9 is a third grade student at El Des-canso Elementary School in Camarillo, California. Amanda was diagnosed with FA in 1986 and as of June, 1989 was "holding her own".

Lynnette has volunteered to be a regional coordinator for "LIFE-SAVERS Foundation of America". This group is actively involved in recruitment of unrelated marrow donors. LIFESAVERS is a contract affiliate of the National Marrow Donor Program and it deserves your full support in our marrow donor recruitment effort.

Urge your family, friends and supporters to enroll as marrow donors. If they cannot be HLA tested themselves, ask them to lend financial support for those who will volunteer to be enrolled. The HLA testing cost has been reduced to \$75.00 per donor. For more information, the toll free number is 1-800-999-8822.

We are aware of a recent successful unrelated matched FA transplant in London. Another FA family as of this writing is undergoing (with our prayers) an unrelated transplant procedure in Iowa. We owe it to all victims of killer diseases of the blood system to expand the pool of willing,

non-family donors. Thank you, Lynnette, for your special efforts to expand the pool of "Good Samaritans" as lifesaving marrow donors.

## **CHANGES IN NAME, ADDRESS OR PHONE NUMBER**

1. Brad & Lea Ann Curry  
1043 Oakridge Drive  
Lanesville, Indiana 47136
2. Stephen & Maureen Dodd  
26 Carlan Drive  
General Delivery  
Port Perry  
Ontario L0B 1N0  
Canada  
416-985-2049
3. Ida Catherine Hodge  
Rt. # 3  
Box 500  
Shepherdsville, Ky. 40165
4. Gayle Licari  
203 Shepard St.  
Havelock, N.C. 28532  
919-444-1189
5. Therese & Terry Robertson  
7115 42nd St.  
Court North  
Oakdale, Minn. 55128  
612-779-2393
6. Dejuana Simon  
2325 Boxwood Ave, #4  
Sacramento, CA 95815
7. Walter & Tina Smith  
95009 Waikalani Drive A110  
Mililani, Hawaii 96789

8. Vicki & Gordon Ware have moved  
Does anyone know their present  
address?

9. Sandy & Marc Weiner (add phone #)  
914-232-0385

## **MEDICAL POINTERS FOR FA PATIENTS AND PHYSICIANS**

Blanche Alter, M.D. is a leading pediatric hematologist. Her research into bone marrow failure syndromes is widely recognized. Dr. Alter, whose efforts are partially supported by the Fanconi Anemia Research Fund, Inc., offers some important medical pointers for FA families:

1. Transfusions: Any blood products should be obtained only from unrelated donors. Family members should not be used, to decrease the risk of sensitizing the patient. If there is an HLA matched family member who may be used as a bone marrow transplant donor, that person must not be a donor for blood products prior to transplant. If an unrelated transplant is being considered, family members should still not be donors of blood products because of potential sensitization.

2. Bone marrow suppressive agents: Drugs and toxins which might perhaps cause acquired aplastic anemia in non-FA persons should be avoided by FA patients if possible: Chloramphenicol, phenylbutazone, sulfonamides, anticonvulsants, gold, benzene, organic solvents (eg. paint thinner, airplane glue), insecticides.

3. Platelet Function: Certain drugs might interfere with platelet function, and thus



increase bleeding or bruising in patients whose platelet count may already be low. These include aspirin, nonsteroidal antiinflammatories (Inderal, Advil), many antihistamines, glycerol guaiacolate, Vitamin E, and cod liver oil. However, if a patient has an allergic reaction to a blood product, he should be treated with antihistamines if needed.

4. Recent analyses of FA patients in the medical literature and in the Registry suggest that several types of problems may be more common than originally thought:

- Gastrointestinal, including narrowings or digestive problems.
- Kidney, including subtle infections, and abnormalities of ureters.
- Hearing may be decreased, and should be monitored to ensure proper speech development and learning.

Families should bring the above items to the attention of their physicians if relevant.

Blanche P. Alter, MD  
The Mount Sinai Medical Center  
New York, New York  
November 3, 1989

## **FAMILIES CONTINUE TO GENERATE NEEDED FUNDS FOR RESEARCH**

Since our fundraiser began in February, 1989, thirty-seven FA families have generated an incredible \$315,000.00 for FA research. We all need to take a great deal of pride in this accomplishment.

- Bob and Linda Scullin have sent many letters to friends and business associates, and have raised over \$30,000 for FA. This

effort has consumed much of their time for the last few months, and the results are truly impressive. We are deeply grateful for their wonderful help.

- Jackie and Bill Lucarell continue their many innovative fundraisers, including a Cut-A-Thon which needed \$2200 for research. They have now raised over \$25,000 for our Fund. See page 7 for additional details concerning their successful, creative fundraising efforts.

- Lynn and Dave Frohnmayer have raised over \$185,000 for research and to offset costs of a weekend workshop on FA (see article on page 1). Since the Fund received its 501 (c)(3) status, it is eligible for grants through various foundations. The Frohnmayers plan to write several grant proposals in the near future and will report on the success of these efforts in the next newsletter.

- Other families have continued to raise research funds since our last newsletter. Our deepest thanks to the following families for their on-going efforts: Therese and Terry Robertson, Diane and Matthew Senatore, Margaret and Brian Curtis, Alice and Robert Nicholson, Marlene Stone and Robin Paulson, Lorraine and Kevin O'Connor, Hal and Bobbie Porter, Durant and Ridgely Worthy, Sandy and Marc Weiner, Lester and Phyllis Resh, Nancy and Reese Williams, Vicki Phillips, Byron and Denise Adamson and Ida Hodge. WE NEED TO WORK UNTIL THERE IS A CURE FOR FA. PLEASE CONTINUE YOUR WONDERFUL EFFORTS.





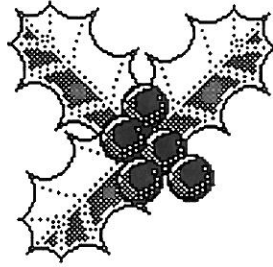
## **ADDITIONAL FUNDRAISING EFFORTS TO SUPPORT RESEARCH**

Several FA families have solicited research funds and given directly to a specific researcher known to them. John and Barbara Miller raised \$1,150.00 to assist Dr. N.T. Shahidi's research at the University of Wisconsin Hospital and Clinics. Ed and Janice Duffy raised \$2325.00 which they sent directly to Dr. Arleen Auerbach at The Rockefeller University. These efforts have greatly assisted our joint cause. Many thanks for your help!

## **FUNDS RAISED FOR FAMILY SUPPORT**

A specific objective of the Fanconi Anemia Research Fund, Inc. is "to provide support services to Fanconi Anemia patients and their families." You should be aware of this avenue of financial support.

The Fund was able to accept tax-deductible donations raised on behalf of the Curry family prior to Natalie's transplant. The Fund, in turn, made a grant that covered the otherwise uninsured costs of the Curry family's stay in Paris, France while Natalie recovered. Please let us know if the Fund can assist you in a similar way.



**HAPPY  
HOLIDAYS!  
  
TO ALL  
  
OF OUR  
  
FAMILIES**