Welcome to the fifteenth issue of the FA Courier, outlining current requests for materials and clinical trials connected to Fanconi anemia (FA) research. We publish the FA Courier:

- To encourage families to contribute to the urgent need for research materials necessary for FA research;
- To keep families informed about current research projects and clinical trials; and
- To apprise researchers of the availability of research materials.

Thank you for helping to advance Fanconi anemia research.

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For FA Patients and Families

Please Support FA Research by Donating Research Materials

FA researchers are in need of biomedical research materials such as blood, bone marrow, skin (fibroblasts), samples of tumors and related medical records. To help collect these materials, the Fund is partnering with the National Disease Research Interchange (NDRI). If you are interested in donating research materials to NDRI, please contact the Private Donor Program by email at privatedonor@ndriresource.org or by phone at 1-800-222-6374. For more information or assistance in donating tissue, contact Teresa Kennedy, the Fund’s Director of Family Support Services, at 1-888-FANCONI or teresa@fanconi.org.

Etiologic Investigation of Cancer Susceptibility in Inherited Bone Marrow Failure Syndromes

This project will identify cancer-prone families with underlying Fanconi anemia prior to the appearance of cancer.

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Etiologic Investigation of Cancer Susceptibility in Inherited Bone Marrow Failure Syndromes

Funding source: National Cancer Institute, Intramural Research Program

Accepting: International patients

Hypothesis:
This project will identify cancer-prone families with underlying Fanconi anemia (FA) prior to the appearance of cancer. The goal is to learn more about FA, in order to improve the quality of life for persons from affected families. Hypotheses: 1) A prospective cohort will provide new information regarding cancer risk. 2) Mutation in FA genes are relevant to cancer pathways in non-hereditary forms of cancers. 3) Patients with FA who develop cancer differ from patients with FA who do not develop cancer. 4) Carriers of FA mutations are at an increased risk of cancer. 5) A substudy will explore the experiences of healthy siblings of FA patients, in order to determine how we can help families manage FA.

Importance of project to FA patients:
FA patients have a remarkably high risk of leukemia and solid tumors. A large epidemiologic study will determine actual cancer risks, identify individually predictive features and define management. The prognostic significance of specific FA mutations and non-FA genes will be identified. The role of viruses in FA solid tumors will be examined. Features of the bone marrow that are associated with progression to leukemia will be defined. FA patients are at high risk of HPV-associated head and neck and gynecologic cancer.

Eligibility criteria:
1) Any patient with FA. Bone marrow failure is NOT required. 2) Patients with suspected FA despite negative chromosome breakage tests. 3) First-degree relatives: siblings (half or full), biologic parents, biologic grandparents and children. 4) Non-FA patients with tumors of the types seen in FA (head and neck, esophageal and gynecological), without the usual risk factors (e.g., age, smoking, drinking).

Material/Information needed:
Questionnaires: Family History Questionnaire (in-depth family medical history); Individual Information Questionnaire (in-depth personal medical history for the patients and their immediate family members); Follow-up Form (every 2 years). Bone marrow: 2-5 ml of marrow, marrow aspirate and biopsy slide. Tumor tissue: fresh, reports, slides, blocks. Blood, serum and plasma samples. Mouth washings for oral cavity cells. Skin biopsies for chromosome breakage or DNA (in some patients). Results of gynecologic exams (females). All participants contribute personal medical and risk factor information and often samples of blood (bone marrow for those with FA) from their home community. Some families will visit the NIH Clinical Center for a more comprehensive clinical and laboratory evaluation. FA mutation testing will be performed in a CLIA-certified laboratory. Sibling interviews will be done at home or at the NIH.

Cost of participating:
All costs for participating, including transportation (from US and Canada), hotel and meals for all family members, will be paid by the NCI.
The International Fanconi Anemia Registry

Investigating the correlation between genotype and phenotype will define important regions within the FA genes that may shed light on their function in cell cycle control, programmed cell death and DNA repair.

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Funding Source: The Rockefeller University

Accepting: International patients

Hypothesis:
The Rockefeller University Hospital is home to the International Fanconi Anemia Registry (IFAR), established in 1982 to study a large number of patients exhibiting the full spectrum of diverse features of FA. Questions relating to diagnosis, natural history of the disease, prognosis, treatment and cancer incidence in FA are being addressed by the IFAR studies. Information regarding genotype-phenotype correlation is being obtained, which may help to determine the physiologic roles of the cloned genes. We hypothesize that correlation between genotype and phenotype will define important regions within the FA genes that may shed light on their function in cell cycle control, programmed cell death and DNA repair. This may lead to improvement in prediction of outcome for a given patient, based on genotype, and affect decision-making regarding timing of therapy options.

Importance of project to FA patients:
We aim to more fully define the variable clinical manifestations associated with FA, particularly the congenital malformations and malignancies, and to determine to what extent the clinical findings in FA patients and carrier family members correlates with the specific mutation/region of mutation, i.e. genotype. The recent identification of the genes responsible for ~90% of the cases of FA make it possible to evaluate patients and family members by mutation group, comparing phenotype with genotype. We will conduct a thorough clinical and molecular genetic analysis with the objectives of learning about the extent, the causes and the optimal treatment for FA-associated medical problems. As part of the project, we are developing more rapid methods for mutation screening. Genetic information will be made available to patients’ physicians as appropriate by law.
The International Fanconi Anemia Registry continued

Eligibility criteria:
Any patient diagnosed as affected with FA, as well as parents.

Material/Information needed:
We need to receive a blood specimen from the patient to make a cell line. Mutation testing will be performed in a research laboratory. The IFAR form must be completed and can be downloaded at: www.rockefeller.edu/labheads/auerbach/documents/IFAR_FORM.pdf.

Cost of participating:
The patient’s blood and other specimens are usually shipped by clinician, as part of clinical testing.

Needed: Blood sample; bone marrow

Laboratory Studies of Gene Transfer for Fanconi Anemia

In order to optimize the delivery of a normal FANCA or FANCC gene to abnormal cells, and to test its ability to correct the Fanconi anemia defect in the laboratory, researchers need a source of bone marrow and/or blood from patients with FANCA or FANCC.

Principal Investigator: Pamela S. Becker, MD, PhD
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Funding Source: National Institutes of Health (National Heart Lung and Blood Institute)

Accepting: International patients

Hypothesis:
The long-term goal of our research is to develop gene therapy as a treatment option for patients with Fanconi anemia. The first steps in this effort are to develop effective methods to deliver a normal gene to the blood stem cells of patients with Fanconi anemia. At this time, we are focusing on Fanconi anemia complementation group A (FANCA) and Fanconi anemia complementation group C (FANCC). We have developed a genetically modified, non-disease-causing virus that can deliver the FANCA or FANCC gene to blood cells in a laboratory dish. We have tested it in blood cells from people who do and do not have Fanconi anemia, and in blood cells from mice with Fanconi anemia. In order to optimize the delivery of a normal FANCA or FANCC gene to abnormal cells, and to test its ability to correct the Fanconi defect in the laboratory, we need a source of bone marrow and/or blood from patients with FANCA or FANCC.

Importance to FA patients:
Optimizing the gene delivery process is an essential step in developing gene therapy as a safe and effective treatment option for Fanconi anemia patients.

Eligibility criteria:
- Fanconi anemia complementation group A or C as determined by somatic cell hybrids, molecular characterization, Western blot analysis or acquisition of mitomycin C resistance after in vitro transduction with a vector bearing the cDNA for Fanconi complementation group A or C
- Undergoing bone marrow aspiration and/or blood draw for clinical purposes
Laboratory Studies of Gene Transfer for Fanconi Anemia

**Material needed:**
10 mL (2 teaspoons) of blood and/or 5 mL (1 teaspoon) of bone marrow

**Cost of participating:**
There is no cost for participating. Dr. Becker's laboratory will pay for shipment.

### Phase II Study of Single-Agent Cetuximab for Treatment of High-Risk Pre-Malignant Upper Aerodigestive (UAD) Lesions

This is a randomized trial of cetuximab treatment for patients with high-risk, premalignant UAD lesions. Patients will also be followed for development of head and neck cancer. Following the eight-week treatment with cetuximab, assigned groups will undergo lesion resection based on the extent of initial disease.

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**Accepting:** International patients

**Rationale:**
Over 45,000 new cases of Head and Neck Squamous Cell Carcinomas (HNSCC) are diagnosed in the United States yearly, and this disease affects over 600,000 people worldwide. The data identify a set of high-risk patients with oral pre-malignant lesions as a population with extraordinary risk for malignant progression and significant mortality, for whom there is no viable medical or surgical intervention. Cetuximab is an attractive therapeutic agent for these patients, in that it has a low risk toxicity profile and is effective in invasive head and neck cancer.

**Purpose:**
This is a randomized trial of cetuximab treatment for patients with high-risk, premalignant UAD lesions. Three groups of patients (group 1: diffuse, group 2: recurrent, group 3: dysplastic) will receive cetuximab 400 mg/m2 on week one followed by 250 mg/m2 on weeks 2-8. Patients in the control arm will have the option of moving into a treatment arm after completion of initial treatment. Patients will also be followed for development of HNSCC. Following the eight-week treatment with cetuximab, groups 2 and 3 will undergo lesion resection based on the extent of initial disease. Safety of cetuximab in this patient population will also be evaluated. The projected accrual goal is a total of 60 patients. 20 of the 60 patients are targeted to be enrolled at the Coordinating Center at Johns Hopkins, while enrollment of 40 patients is to be completed at 11 participating sites. IRB approval has been obtained and enrollment has started at Johns Hopkins, Medical University of South Carolina and University of Illinois, Chicago, while other participating site are in the process of obtaining IRB approvals. To date, a total of 17 patients have been enrolled into the study, 6 have been treated, 2 are under treatment and 2 patients are in screening.
Phase II Study of Single-Agent Cetuximab for Treatment of High-Risk Pre-Malignant Upper Aerodigestive (UAD) Lesions

The inclusion criteria for enrollment are:
Histologically confirmed, previously untreated, high-risk UAD pre-malignant lesion consisting of one of the following groups:

- Unresectable, diffuse high grade dysplasia, defined as moderate or severe dysplasia whose anatomic extent cannot be assessed by physical examination and/or includes a large enough area or area of anatomic extent that cannot practicably be excised by standard surgical techniques
- Previously treated HNSCC with persistent or recurrent high grade dysplasia with no evidence of head and neck malignancy for three months prior to enrollment
- Dysplastic lesions with 3p or 9p loss of heterozygosity

Patients will undergo the following after enrollment into the study:
1. Informed consent
2. Complete Head and Neck Exam
3. Tolonium staining and Photodocumentation:
   a. Tolonium Staining using vizlrite Blue Oral Exam Kit - this is to be provided free of cost
   b. Photodocumentation
4. Biopsy (3mm punch) of lesion OR prior biopsy less than 3 months prior to enrollment
5. Tumor specimen for LOH testing
6. Pre-treatment evaluation which includes:
   a. Blood tests to confirm eligibility to receive study drug and
   b. Imaging studies to assess extent of disease as per the standard of care for the patient. Research blood samples to be sent to Coordinating Center.
7. Randomization to one of the two following groups:
   a. Study Drug Group: Patient to receive weekly cetuximab injections for eight weeks on Days 1, 8, 15, 22, 29, 36, 43 and 50.
   b. Control Group: To undergo scheduled follow-up and observation on the same days mentioned above. Patients in this group may choose to receive the study drug after the eight-week period.
8. Post Treatment:
   a. Head and Neck Exam and blood draw
   b. Tolonium Staining and Photodocumentation:
      • Tolonium Staining using Vizlite Blue Oral Exam Kit - this is to be provided free of cost
      • Photodocumentation
   c. Repeat biopsy of tumor for Group 1, and/or excision of lesion for patients in Groups 2 and 3
   d. Repeat biopsy specimen to go for LOH testing
Phase I/II Dose Escalation Trial of Danazol in Patients with Fanconi Anemia or Dyskeratosis Congenita

The purpose of this Phase I/II dose escalation trial is to determine the minimum effective dose of danazol, an attenuated androgen, when used for Fanconi anemia and Dyskeratosis congenita and to evaluate adverse side effects.

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Accepting: International patients

Hypothesis:
Fanconi anemia (FA) and Dyskeratosis congenita (DC) are inherited bone marrow failure syndromes for which anabolic steroids are often used, but in which specific androgens have never been studied. Masculinizing side effects from the usual androgen (oxymetholone) present major problems, leading to a need for another agent. Danazol is an attenuated androgen, and thus may have fewer side effects; however, its hematologic efficacy in the setting of FA and DC has never been investigated.

The purpose of this Phase I/II dose escalation trial is to determine the minimum effective dose of danazol and to evaluate adverse side effects. An additional goal is to investigate gene expression signatures of patient progenitor cells after exposure to danazol, both in vitro and in vivo, to correlate gene expression with responsiveness to treatment and to identify new treatments.

Importance of project to FA patients:
If danazol does not have serious masculinizing or other side effects and is efficacious in stimulating blood cell production, it could prove very useful in the management of bone marrow failure, particularly among patients who do not have a matched sibling donor.

Eligibility:
Inclusion criteria:
• Patients must be diagnosed with FA that is documented by a positive chromosomal breakage test
• Patients must have at least one of the following peripheral blood cytopenias: Absolute neutrophil count <500 µL; platelet count <30,000/µL; hemoglobin <8.0 gm/dL
• Patients must have a negative pregnancy test and agree to use medically approved birth control
• Patients must be either three years of age or ≥14 kg (30 lbs)
Exclusion criteria:
• Concurrent use of anticoagulants
• Use of androgen therapy within past three months
• Patients with liver disease (SGOT, SGPT or bilirubin greater than the upper limit of normal)
• Patients with renal disease (serum creatinine greater than the upper limit of normal for age)
• Patients who have HLA matched sibling donors
Phase Ib Study of Erlotinib Prior to Surgery in Patients with Head and Neck Cancer

In this program, patients with head and neck cancer who are candidates for surgical resection receive therapy with erlotinib, a novel molecular-targeted drug, during the period leading up to surgery. The post-treatment surgical specimen is then collected and the tumor is analyzed for changes in cancer cell molecules in response to therapy.

Principal Investigator: William N. William Jr., MD
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Purpose of Study:
A major area of interest in head and neck cancer research has been the development of treatment based on molecular targeted agents. These drugs have unique mechanisms of actions and are generally less toxic than traditional chemotherapy. Despite the increased use of molecular targeted agents for treatment of head and neck cancers and other cancers, little is known about which patients are more likely to benefit from these drugs, and how cancer cells become resistant to treatment. In an attempt to better understand the biology of head and neck cancers and how they respond to targeted agents; at MD Anderson we have developed a molecular-based, pre-operative treatment program for patients with head and neck cancers who are candidates for surgical resection.
Phase Ib Study of Erlotinib Prior to Surgery in Patients with Head and Neck Cancer

Eligibility criteria:
Patients with head and neck cancers who are candidates for surgical resection. FA patients are not excluded.

What is involved?
In this program, patients receive therapy with a novel molecular targeted drug during the period leading up to surgery. The post-treatment surgical specimen is then collected and the tumor is analyzed for changes in cancer cell molecules in response to therapy. The first molecular targeted drug to be evaluated in this pre-operative program is erlotinib, an oral inhibitor of the epidermal growth factor receptor. This receptor is present on most head and neck cancer cells and enables them to survive and proliferate, thus contributing to tumor growth and spread. Erlotinib is already FDA-approved for treatment of lung and pancreatic cancers at the doses of 100 - 150 mg/day. However, pre-clinical data suggest that higher doses of erlotinib may be more effective in killing cancer cells. Therefore, patients participating in the study are randomized to receive the standard dose or a higher dose of the drug for two to eight weeks prior to surgery.

What are the benefits?
Since erlotinib is well tolerated, the study allows for enrollment of a broad patient population. So far, more than half of the planned number of participants has been accrued, with promising preliminary results. Additional pre-operative studies, with drugs directed at other molecular targets, will soon be activated at MD Anderson to replace the erlotinib trial. This platform of pre-operative studies allows for patients to receive novel treatments while their surgery is being planned, and provides a rich repository of tumor specimens that will enable us to understand the effects of these treatments on cancer cells, ultimately leading to the design of more effective and less toxic therapies for head and neck cancer.

Nonmyeloablative Hematopoietic Cell Transplantation for Patients with Fanconi Anemia Using Alternative Marrow Donors

The purpose of this study is to test whether it is possible to use an alternative bone marrow donor who is half-matched, or haploidentical, to the patient’s tissue type. Researchers will test whether using lower doses of radiation and a novel immunosuppressive regimen will allow the patient to accept the new donor cells with fewer side effects. This is a dose-finding study. Different doses of radiation will be tested.

Principal Investigator: Hans-Peter Kiem, MD
Fred Hutchinson Cancer Research Center, Seattle, Wash.
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Accepting: US patients only

Contact: Michelle Bouvier, RN
Clinical Trials Nurse, Nonmyeloablative Transplants
Telephone: 206-667-6993
Email: meb@fhcrc.org

Clinical Trial: Half-matched bone marrow
What is the purpose of this study?
Many people with Fanconi anemia develop bone marrow failure or leukemia. Both of these conditions may be cured by stem cell transplantation, but it is sometimes difficult to find an appropriately matched donor to use. The purpose of this study is to test whether it is possible to use an alternative bone marrow donor who is half-matched, or haploidentical, to the patient’s tissue type. The haploidentical donor is related to the patient and is usually the parent or sibling. We can also use matched unrelated donors on this protocol. We will test whether using lower doses of radiation and a novel immunosuppressive regimen will allow the patient to accept the new donor cells with fewer side effects. This is a dose-finding study. Different doses of radiation will be tested.

Who can participate?
This study is open to FA patients who have bone marrow involving two of the following three lineages:

• granulocyte count <0.5 x 10⁹/L
• platelet count <20 x 10⁹/L
• hemoglobin <8 g/dL

or to FA patients with one of the above and a life-threatening event or to FA patients requiring red blood cell or platelet transfusions because of bone marrow failure.

If being treated for leukemia, the patient must be considered in remission.

What is involved?
Patients will undergo a bone marrow transplant using a novel, dose-finding conditioning and immunosuppressive regimen. Treatment will last 3½ months or more, with follow-up every year thereafter.

What are the benefits?
For FA patients who must undergo stem cell transplant for bone marrow failure or leukemia, but do not have a fully matched donor, this will allow the opportunity for a potentially life-saving treatment. This will also allow the opportunity to use lower doses of radiation therapy to lower the risks of long-term side effects.

What are the risks?
Risks associated with bone marrow transplant are extensive and include infections, graft-versus-host disease, rejection, secondary cancers and even death. Using a half-matched donor or unrelated donor (instead of a fully matched sibling donor) may increase these risks. The risks of this study will be explained in detail to you.

Will you be paid to participate in this study?
No.

To FA Families:
If you need assistance researching a clinical trial or with the costs associated with participating in a clinical trial, please contact Teresa Kennedy, Director of Family Support Services at the Fund. Call toll-free 1-888-FANCONI or email teresa@fanconi.org.
For FA Researchers

Sharing Research Materials:
The Fund requires that all investigators who publicize their need for research materials in the FA Courier share residual research materials received through the FA Courier and cooperate to the maximum feasible extent with other scientists who are studying Fanconi anemia. Read more about the Fund’s requirements at www.fanconi.org.

Available Research Materials

Biomedical samples collected from Fanconi anemia (FA) patients are available to researchers. Given the rarity of FA, the supply of some biomedical research materials is occasionally limited. Samples are provided only to those researchers who are specifically studying FA. The Fund is partnering with the National Disease Research Interchange to facilitate sample collection and distribution. Visit www.ndriresource.org to learn more.

Background:
To facilitate collection of biomedical research materials for FA research, the Fanconi Anemia Research Fund has entered into a partnership with the National Disease Research Interchange (NDRI). NDRI is a 501(c)(3) not-for-profit organization with over 25 years experience distributing human cells, tissues and organs to researchers and scientists (www.ndriresource.org). In conjunction with the NIH Office of Rare Diseases, in 2002 NDRI began to develop a program focused on the unmet needs of the rare disease research community for human organs and tissues. NDRI receives funding for its Rare Disease Program from the National Institutes of Health and the Office of Rare Diseases.

Available Research Materials:
Biomedical samples of Fanconi anemia are available to researchers. Given the rarity of FA, the supply of some biomedical research materials is occasionally limited. Samples are provided only to those researchers who are specifically studying FA.

In general, NDRI places around 20,000 biospecimens annually with researchers in academic and government laboratories, as well as those in biomedical and pharmaceutical industries. To that end, the organization works with organ procurement organizations, eye banks, tissue banks and major medical centers in the United States. NDRI has also developed unique programs to serve researchers studying rare diseases, such as Fanconi anemia. Importantly, NDRI has developed mechanisms to aseptically obtain tissues with a very short death-to-preservation interval, making them ideal for cell culture, genomic and proteomic research. NDRI also serves the cancer research community with tumor and normal adjacent tissues specimens preserved at 4ºC, frozen, snap-frozen or formalin-fixed/paraffin embedded.

NDRI will strive to meet your exact needs for FA research. Please visit www.ndriresource.org to learn how NDRI can be a valuable resource for your research. To receive an application, contact a Rare Disease Coordinator at raredisease@ndriresource.org or by phone at 1-800-222-6374. The application is also available on NDRI’s website under the Quick Links section.

FA Antibody Project: Antisera Now Available Against Fanconi Anemia Complementation Group Proteins

FARF has sponsored the development of affinity-purified rabbit polyclonal antisera against the Fanconi anemia complementation group proteins in order to facilitate research into Fanconi anemia. Investigators requesting antisera must complete a request via the website www.ohsu.edu/fa.
FA Antibody Project: Antisera Now Available Against Fanconi Anemia Complementation Group Proteins

Background:
The Fanconi Anemia Research Fund has sponsored the development of affinity-purified rabbit polyclonal antisera against the Fanconi complementation group proteins in order to facilitate research into Fanconi anemia.

These rabbit antisera were developed with a commercial partner (Open Biosystems in Huntsville Alabama) using peptide epitopes. The antisera have been validated by a combination of peptide ELISA assays and in many instances Western blot analyses using patient-derived cell lines or with and without peptide blocking. All successful antisera are available affinity-purified, and in many cases as unpurified sera for investigators wishing to perform a specific, alternative purification (e.g., against full-length protein).

The Fanconi Anemia Research Fund has contracted with Oregon Health & Science University through the laboratory of Markus Grompe, MD, Department of Medical and Molecular Genetics, to manage the FA Antibody Project in conjunction with the FA Cell Repository already housed at OHSU, where Laura Marquez is in charge of distribution.

Availability: Affinity-purified antisera are currently available against the following proteins: FANCA, B, C, D1/BRCA2 and D2, E, F, G, J, L, M and N, and the deubiquitinating enzyme USP1 protein. FANCI antisera directed against three different epitopes is currently in production and should be ready for distribution in Fall 2010. For a current listing of available antisera please check the FARF Antibody Project website: http://www.ohsu.edu/fa. These reagents are available without charge to qualified investigators willing to pay shipping (see below).

IMPORTANT: An essential part of this Antibody Project is sharing experience using these antisera with the Fanconi community. Thus, all end users need to report their experience on the best protocols and uses of specific antisera for immunofluorescence, immunoprecipitation, Western blot or other analysis to help others to best use these antisera. This feedback can be e-mailed or entered on the Antibody Project website. A current compilation of user data can be downloaded from the website as well. We will not honor additional requests for reagents from users who do not provide feedback on their experience using antisera.

How to obtain antisera:
Investigators requesting antisera must complete a request via the website www.ohsu.edu/fa. This includes providing information on intended use in sufficient detail to allow us to assess whether the request fosters FA research; completion of the Use Agreement and Materials Transfer Form; and provision of a valid shipping account number to cover the cost of shipping. Commercial users may arrange to purchase the same antisera via a distribution agreement with Open Biosystems.

Questions?
Direct general inquiries regarding FANC antibodies and the Antibody Project to:
Ray Monnat, MD
Departments of Pathology and of Genome Sciences
University of Washington
Telephone: 206-616-7392
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Direct antibody distribution questions to:
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Pilot Study Awards: Funding Available for Research

The Fanconi Anemia Research Fund, Inc. seeks applications for basic or applied research into the mechanisms, pathogenesis and/or treatment of Fanconi anemia. These awards are usually for one year of support and are intended to fund pilot studies designed to test new ideas and to provide initial data to support applications for further funding by other (and larger) agencies that support biomedical research.

Fanconi anemia is an autosomal recessive disease characterized by bone marrow failure, variable congenital anomalies and a predisposition to leukemia. Cells from FA patients exhibit hypersensitivity to alkylating agents such as mitomycin C (MMC) and diepoxybutane (DEB). Indeed, the hypersensitivity to cytotoxic effects of DNA cross-linking agents is currently used as the basis for the diagnostic tests for FA. It is known that FA is genetically heterogeneous, with at least thirteen complementation groups and thirteen cloned genes [A, B, C, D1 (BRCA2), D2, E, F, G, I, J, L, M, and N] identified thus far.

Better understanding of Fanconi anemia that can lead to effective treatment of the various phases of the disease is so urgently needed. Studies of Fanconi anemia may also have important implications for solid tumor malignancies such as head and neck, gastrointestinal, and gynecological cancers.

Application Process:
An abbreviated NIH-style application is used. Under emergency circumstances researchers whom we are currently supporting or who have an established track record in FA research can apply for small supplementary grants on an accelerated basis. Selection for a research award is based upon scientific merit and relevance, as determined by the Fund’s peer review procedure. Applications are reviewed on an ongoing basis.

Apply to:
Fanconi Anemia Research Fund
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