Spring 2005

DBA Newsletter

Volume 7, Number 1 • When we all work together, anything is possible!

DBAF Funds Research Projects Totaling $436,692 in 2004

The Diamond Blackfan Anemia Foundation, Inc. (DBAF) is pleased to report the funding of research grants totaling over $436,000. This research is made possible by our families' dedication and commitment to our goals. As many of these projects are multiple year projects, and an increasing number of experienced researchers are becoming interested in conducting DBA research, we are hoping more families will help us continue to fund future approved research proposals. The DBAF is proud to be able to attract and support the research projects of world-renowned physicians and researchers. We need to work together to insure funding for all worthwhile projects. The DBAF is grateful to both the researchers who have dedicated their time to study DBA and our families who continue to recognize the importance of their research.

Dr. Gil Tchernia of Bicetre Hospital, France received $19,000 as the second year funding of the scientific grant titled "Mechanistic Understanding of DBA Pathophysiology". The long-term goal of this research project is to decipher the mechanistic understanding of the pathophysiology of DBA, and more specifically, to evaluate the role of ribosomal protein S19 (RPS19) in erythropoeisis and then to define the link between the mutation in RPS19 gene and the occurrence of the disease. New insights generated by this study could help design new therapeutic strategies for DBA patients.

The DBAF co-sponsored the Fifth Annual Diamond Blackfan Anemia International Consensus Conference with the Daniella Maria Arturi Conference for $40,000. A separate article detailing the highlights of the conference can be found in this newsletter.

A grant for $70,771 was awarded to Mahmut Y. Celiker, MD, Jeffrey Lipton MD, PhD, Steven Arkin, MD, and Adrianna Vlachos, MD, of Schneider Children's Hospital, New Hyde Park, NY for the second year of a project titled "Gene Expression During Erythropoeisis in DBA". The long-term objective of this study is to apply the information gathered about the pathophysiology of DBA, to develop new treatment modalities, to optimize the current treatment methods, and eventually bring a cure to all DBA patients. Research findings from the first year of this study, also funded by the DBAF, have been submitted for publication in a very prestigious journal, "Experimental Hematology".

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Dr. Sarah Ball of St. George's Hospital Medical School in London, England was the recipient of a $78,283 grant for her project titled "Enhancement of Steroid Response in DBA: Application of an in vitro two-phase erythroid culture model". This project will use a peripheral blood in vitro two phase erythroid culture model to study erythroid failure in DBA and the stimulatory effect of steroids. The use of agents that enhance steroid effectiveness and the interactions of other cytokines and hormones (including prolactin) on the production of red blood cells in DBA patients will be investigated, with the aim of identifying potentially therapeutic combinations that enhance the effectiveness of steroids.

The University of Louisville, Kentucky was awarded $26,709 for Steve Ellis PhD to assume the responsibilities of Research Director. In addition to the responsibilities outlined in a separate article in this newsletter, the grant will cover expenses such as a computer and laboratory supplies for related DBA research.

A grant for $106,929 was given to Hanna Gazda, MD, PhD, of Dana Farber Cancer Institute, Boston, MA, for her project "Genetics and Biology of DBA". This investigation will test the hypothesis that detailed clinical and genetic examination of a large number of patients and careful statistical analysis will reveal a phenotype-genotype in individuals with identified RPS19 mutations. Please see Dr. Gazda's article in this newsletter for additional information.

Dr. Niklas Dahl of Uppsala University Children's Hospital, Uppsala Sweden has been awarded $50,000 for the second year of a project titled "Identification of the Molecular Basis of DBA and the evaluation of RPS19 gene transfer". This $165,251 project is also receiving funding from three other Swedish organizations. The long term objective of this study is to better understand the basic molecular pathology behind DBA by identifying factors interacting with RPS19 and/or its messenger RNA, and to develop a novel treatment modality for DBA by studying gene transfer in a DBA mouse model.

A grant of $45,000 was awarded to Stefan Karlsson MD, PhD, of Lund University, Lund, Sweden to assist in the funding of his four-year project titled "Cellular and Murine Models for RPS19 deficient DBA". The goal of this project is to characterize the effects of RPS19 depletion on erythropoiesis using siRNA lentiviral vectors. Please see the related article in this newsletter for additional information.

Steven R. Ellis is Appointed to the Position of Research Director

The DBAF is pleased to announce the appointment of Steven R. Ellis to the position of Research Director. Mr. Ellis is an Associate Professor, Department of Biochemistry and Molecular Biology, University of Louisville, Louisville, Kentucky. He is a well regarded researcher that has worked in areas very relevant to DBA. His research project titled "Ribosome Function and Diamond Blackfan Anemia" was recently approved for funding by the National Heart, Lung and Blood Institute.

As Research Director, Mr. Ellis' long term goals are to maximize the Foundation's efforts to support research relevant to the care and treatment of individuals with Diamond Blackfan Anemia. He will work closely with the Board to establish research goals and develop strategies to achieve them. As Research Director, Mr. Ellis is also responsible for bringing the DBA Board new developments in a range of scientific disciplines relevant to DBA. He will attend scientific meetings in areas that impinge on both clinical and basic science features of DBA in order to allow him to develop and foster contacts with the researchers in the DBA field. This will also help him to identify researchers working in areas related to DBA that may not be presently known to the Board.

We are extremely excited to have Mr. Ellis join our team. His knowledge and experience will help ensure that our research dollars are spent wisely and efficiently.

Mr. Ellis kindly spent a few minutes sharing his thoughts with us about the Diamond Blackfan Anemia Foundation.

What about your background do you think is going to be your greatest asset for DBAF?

Commitment. I've been given an opportunity to take my research background and training and focus it on a very worthwhile endeavor. I am committed to doing the best that I can for the patients and families living with DBA.

At the end of the year, if you had one thing to accomplish what would you hope that to be?

What I would like to see accomplished by the end of the year is the identification of a second gene responsible for DBA. This, I think, is an important goal for the entire research community. At the present time we have a single gene involved in DBA, ribosomal protein S19. Even for a person like me, who thinks about ribosomal proteins all the time, it is difficult to envision how a defective ribosomal protein could give rise to DBA. Identification of a second gene may give further insights into the role of the ribosome or protein synthesis

as a causative agent in DBA or it may direct the DBA community in a very different research direction. There are several laboratories actively working on identifying a second gene for DBA and once it is found I think there will be a tremendous amount of excitement and re-tooling of research efforts to understand the molecular mechanisms underlying DBA.

What do you think that we as parents can do to help DBAF be more effective?

I think the parents are doing an outstanding job. Your efforts in fund raising provide a significant amount of funds to support high-quality research on DBA. These funds not only directly help in understanding the molecular basis of DBA, improving diagnostics, and in the development of new therapies, but also make laboratories more competitive as they apply for federal research dollars. The recent request for applications from the National Institutes of Health for research on Molecular Mechanisms Underlying Diamond Blackfan Anemia and other Congenital Bone Marrow Failures is an example of a federal initiative to support research on DBA. Grants awarded to investigators through the DBAF often allow laboratories to obtain data which strengthen their applications in the intense competition for federal research dollars. So I can't say enough about the parents' effort in this regard. I also thank the parents for allowing me into their lives, if for only a short while, at Camp Sunshine this past year. Getting to know you on a more personal level has only strengthened my commitment to do my best as the research director.

DBA - Related Grants Funded by the National Institutes of Health in September of 2004

These grants were funded through a request for applications from the National Heart, Lung, and Blood Institute for proposals on the molecular mechanisms underlying Diamond Blackfan Anemia and other congenital bone marrow failure syndromes. Data were obtained from CRISP (Computer Retrieval of Scientific information on projects): http://crisp.cit.nih.gov/crisp/crisp_query.generate_screen. Search term: Diamond Blackfan Anemia

Principal Investigator: Dr. Jeffrey M. Lipton, North Shore-Long Island Research Institute
Title: The DBA Registry: A Vital Tool for the Study of DBA
Summary: The goal of this study is to expand and update the DBA registry, a comprehensive database of patients with DBA. The DBA registry is a critical tool that will facilitate investigations into the epidemiology and biology of DBA. The registry will also serve as an important resource to patients and physicians guiding diagnosis, treatment options, and reproductive decisions.

Principal Investigator: Dr. Niklas Dahl, Uppsala University
Title: Molecular Basis of Diamond Blackfan Anemia
Summary: The goal of this study is to obtain a better understanding of the molecular pathology underlying DBA and to develop a novel strategy for treating DBA. Studies on the underlying pathology of DBA will focus on the synthesis and function of ribosomal protein S19. A mouse model for DBA will be used to develop gene therapy as a therapeutic option for DBA patients.

Principal Investigator: Dr. Steven Ellis, University of Louisville
Title: Ribosome Function and Diamond Blackfan Anemia
Summary: The goal of this proposal is to study the role of RPS 19 in ribosome synthesis and function. A detailed understanding the function of RPS 19 can be used to develop improved diagnostic procedures and also lead to the identification other genes involved in DBA pathogenesis. This proposal will also study the relationship between ribosome synthesis and bone marrow failure in a novel transgenic mouse model.

Principal Investigator: Dr. Thomas Glaser, University of Michigan
Title: Modeling Diamond Blackfan Anemia
Summary: The goal of this proposal is to study the effects of disruption of ribosome synthesis and function on mouse development. A strain of mice has been identified that is defective in a ribosomal protein other than RPS 19. Hematopoietic parameters in this strain of mice will be compared with mice strains defective in RPS 19 to examine the extent to which DBA is linked to a selective effect on RPS 19 or whether defects in other ribosomal proteins can result in related pathologies.

Principal Investigator: Dr. Mohadas Narla, New York Blood Center
Title: Diamond Blackfan Anemia and Ribosomal Protein S19
Summary: The goal of this study is to understand the role of RPS 19 in erythropoiesis and how this function is disrupted in DBA patients. RPS 19 levels in erythroid progenitor cells will be experimentally manipulated and the effects on erythroid differentiation examined. Studies will also address the role of prolactin in regulating erythropoiesis and the mechanism of metoclopramide action.

Study to Gather Gene Data for All Families

I work on Diamond-Blackfan anemia (DBA) at the Dana-Farber Cancer Institute, Department of Pediatric Oncology in Boston, MA, in Dr. Colin Sieff's lab. The funding from the DBA Foundation for my grant proposal "Phenotype-Genotype Correlation In Diamond-Blackfan Anemia" has enabled me to screen DNA for ribosomal protein S19 (RPS19) gene mutations from all DBA families from the USA and from outside the country, who want to participate in the study. It also gives me an opportunity to search for relationships between RPS19 mutations and clinical symptoms in DBA. I work with research technician, Ela Latawiec, who pursues blood samples, and with Drs. Donna Neuberg and Shuli Li, the statisticians, who statistically calculate the relationship between RPS19 genotype and DBA phenotype. This project is conducted in collaboration with the Diamon-Blackfan anemia registry run by Drs. Jeffrey Lipton, Adrianna Vlachos, and Clinical Research Coordinator Eva Atsidaftos, who evaluate clinical status and collect blood samples from the patients and their family members.

The first DBA gene, RPS19 identified by Dr. Dahl's group, is mutated in approximately 25% of patients, however its role in DBA remains unknown. The clinical heterogeneity of DBA is still among the most challenging and poorly understood aspects of the disease. Although severe anemia is a prominent feature of DBA, mild anemia or only subtle indications of the red cells abnormality such as increased MCV and/or elevated erythrocyte adenosine deaminase activity are found in some patients. In previous reports the clear correlation between phenotype and a specific mutation was not found. However, a recent study in the UK showed the possibility of phenotype-genotype correlations with respect to hematological severity of anemia and physical anomalies. To further address the question whether there are correlations between genotype such specific RPS19 mutation or lack of these mutations and the clinical symptoms, we undertook more detailed investigation based on larger group of DBA patients.

Our objective is to sequence DNA from at least 400 unrelated DBA patients for mutations in RPS19 gene. We expect to find RPS19 mutations in approximately 100 patients and plan to screen DNA from family members of these patients. We will then investigate the relationship between RPS19 genotype and the clinical symptoms, such as severity of anemia, response to steroid treatment, different physical anomalies, and presence of the malignancy in a family. These relationships may be useful in genetic counseling allowing the prediction of the clinical course of the disease in families with certain mutations. The potential phenotype-genotype correlations may further our understanding of the role of RPS19 protein in erythopoiesis and development and the functional significance of specific RPS19 mutations. Other comparisons will be clinical symptoms in two groups of patients with and without the RPS19 mutations. We will investigate whether there is a statistical difference between these two groups of patients with respect to hematological severity of the disease, physical anomalies, cancer, and evolution of the disease over time.

Finally, we will create DNA and EBV immortalized lymphoblastoid cell line repositories for further studies on DBA.

We are actively seeking patients and their families to participate in this study. Participation will involve review of clinical history and donation of approximately 3-5 ml (2 teaspoons) of blood. Anyone who is interested in participating or in learning more about the study, please contact Dr. Gazda at any time by email (hanna_gazda@dfci.harvard.edu) or by phone (617-632-3258).

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RPS 19 Patients Needed for Gene Therapy Progress

Dr. Stefan Karlsson, a researcher specializing in genetics, has been investigating the possibility of developing gene therapy for patients with DBA who have mutations in the Ribosomal protein S19 gene. In order to continue and accelerate this research, Dr. Karlsson needs more hematopoietic stem cells. Accordingly, he has asked the DBAF to help him identify patients with a deficiency in Ribosomal protein S19 who are willing to undergo peripheral blood stem cell harvest. Dr. Karlsson is looking for patients that are 12Kg or greater in weight. The peripheral blood stem cell harvest is a relatively routine medical procedure. The harvest can be performed at the NIH in Bethesda, Maryland or in Lund, Sweden. **(Continued on Page 5)**

Dear Members of the DBA Foundation:

I am writing to you to solicit your help. During the past four to five years we have been investigating the possibility of developing gene therapy for patients with DBA who have mutations in the Ribosomal protein S 19 gene. The work has progressed well and we have published two recent papers which present evidence for that gene therapy of RPS19 deficient DBA may indeed be feasible. (Hamaguchi et al, Blood, 100: 2724-273 1, 2002 and Hamaguchi et al, Mol Ther 7:613-622, 2003). The progress of the project has gone well scientifically, but we have difficulties obtaining enough hematopoietic stem cells (stem cells that can generate all blood cells) to do the required experiments. Before we can apply to the authorities (FDA and equivalent organizations in countries outside the US) to perform clinical trials to develop gene therapy for DBA, we have to do many additional experiments.

We are now writing to ask the DBA Foundation to help us to identify DBA patients with a deficiency in Ribosomal Protein S 519 who are willing to undergo peripheral blood stem cell harvest to help in the development of gene therapy for DBA. We are looking for patients that are more than 12kg in weight.

We sincerely hope that you are willing to help us to identify patients that want to help us cure DBA.

Best wishes,

Stefan Karlsson MD, PhD
Professor of Molecular Medicine

Johan Richter MD, Phd
Senior Physician

If you are interested in becoming a donor, or would like more information, contact Andy Wagner at figarosail@aol.com

International Consensus Conference Brings Doctors Together From Around the World

By Steven R. Ellis, PhD

A first time participant's review of the 5th Annual Diamond Blackfan Anemia International Consensus Conference sponsored by the Daniella Maria Arturi Foundation and co-sponsored by the Diamond Blackfan Anemia Foundation. The meeting was dedicated to the memory and in honor of Alexandra Reinhardt.

Marie Arturi began the conference by welcoming the participants and announcing that the National Heart Lung and Blood Institute of the National Institutes of Health will initiate a Request for Applications to fund research into the Molecular Mechanisms Underlying Diamond Blackfan Anemia and other Congenital Bone Marrow Failure Syndromes. This was a very exciting development that will substantially increase the amount of funding available for DBA-related research.

The scientific sessions began with a clinical perspective of DBA. Dr. Blanche Alter (National Cancer Institute, Bethesda) gave an overview of DBA and some of the latest developments in attempts to correlate the clinical features of DBA with the underlying genes involved. At present, strict correlations remain elusive. Dr. Sarah Ball (St. George's Hospital, London UK) reported on a population study in DBA patients and their immediate families addressing whether cases of DBA originally thought to have arisen spontaneously were, in fact, genetically transmitted. The data reveal hematological abnormalities in family members without overt DBA, suggesting that the absence of clinically manifested DBA in parents does not preclude the possibility of transmitting DBA to a child. These data have far reaching implications for genetic counseling and selection of sibling bone marrow donors. Dr. Gil Tchernia (on behalf of the French DBA working group) gave two reports; the first on the risk of complications during pregnancies of women with DBA, and the second on the incidence of malignancies in DBA patients within the French registry. Women with DBA included in this study showed a high rate of complications mostly originating from the affected mother, rather than the DBA status of the offspring. It was concluded that pregnancies in women with DBA are high risk and should be carefully monitored. The incidence and type of malignancies observed within the French DBA registry appears to correlate with other population studies previously reported in the scientific literature.

The second scientific session focused on treatments for DBA. Dr. Gil Tchernia, Dr. Samuel Blackman (Cincinnati Children's Hospital, Cincinnati) and Dr. Michael Jeng (Stanford University, Palo Alto) all

gave reports on clinical trials involving the use of metoclopramide and/or recombinant prolactin. The results on the trials with metoclopramide were generally disappointing with some suggestion of adverse effects in the Cincinnati study. Dr. Jeng's study differs from the other two studies by including recombinant prolactin in the clinical trial. It is possible that prolactin, which is induced by metoclopramide, may behave differently from metaclopramide in clinical trials. Dr. Carole Paley (Novartis Oncology) reported on Exjade, an oral iron chelator, developed by Novartis. Exjade has been used in Phase II clinical trials on patients with Thalassemia Major. The data indicate that Exjade is an effective iron chelator with a favorable safety profile that may be useful in treating iron overload in other transfusion-dependent diseases including DBA. The session concluded with an update on the DBA registry of North America by Dr. Adrianna Vlachos (Schneider Children's Hospital, New Hyde Park) who presented data indicating a lack of correlation between DBA patients with cleft palates and mutations in the RPS19 gene. Dr. Vlachos also discussed recent results on hematopoietic stem cell transplantation in DBA. The session ended with a presentation by Dr. Joerg Meerpohl (Zentrum fur Kinderheikunde und Jugendmeizin, Germany) on the network of bone marrow failure syndromes in Germany.

The third scientific session focused on the biology of DBA. Dr. Sarah Ball spoke on a new two-phase system for studying erythroid development to further define the step in erythropoiesis affected in DBA. She went on to show the positive effect of steroids in this two phase system and further reported a synergistic effect of steroids and prolactin in promoting the first phase. Two talks, one by Dr Hanna Gazda (Dana Farber Cancer Institute, Boston) and a second by Dr. Benjamin Ebert (Dana Farber Cancer Institute) discussed using genomic approaches to study whole genome wide gene expression changes in bone marrow cells from DBA patients and cells where the RPS19 expression had been reduced using specific gene silencing technology. Identification of genes whose expression is altered in DBA can provide useful insight into pathways affected, which could lead to improved therapies. Dr. Steven Ellis (University of Louisville) reported on the function of the Rps19 protein in yeast and showed that the yeast protein was required for the synthesis of functional ribosomes. Dr. Gregor Adams (Massachusetts General Hospital) finished the session by discussing how the environment of the bone marrow could influence hematopoietic stem cell development, defining a specific niche which could potentially be manipulated pharmacologically.

On day two of the meeting there was a short session on identifying new genes linked to DBA. Dr. Janis Abkowitz (University of Washington, Seattle) discussed an exciting new candidate gene for DBA which encodes the feline leukemia virus subgroup C receptor. Unfortunately, at the present time there is no data to support the involvement of this receptor in DBA. Dr. Samuel Blackman (Cincinnati Children's Hospital Medical Center) reported on a patient with DBA that had a deletion in chromosome region 3q27. This observation suggests a gene within this region may give rise to DBA.

The final scientific session focused on model systems for the study of DBA. Dr. Leonard Zon reported on the use of zebrafish to study hematopoiesis. His laboratory has identified a minimum of 30 different genes involved in hematopoiesis, some of which may be defective in DBA. Dr. Hans Matsson reported results relating to a transgenic mouse with a defective RPS19 gene created in the laboratory of Dr. Niklas Dahl (Children's Hospital Uppsala University, Sweden). While this mouse does not have the clinical manifestations of DBA, it does have a measurable defect in hematopoiesis, which should provide a valuable resource for gene therapy trials. Dr. Johan Flygare and Dr. Stefan Karlsson both from Lund University in Lund Sweden spoke on mouse models where RPS19 gene expression was reduced using siRNA technology. This approach was shown to disrupt erythroid differentiation indicating that it may be a valuable tool for studying the role of the Rps19 protein in DBA. The final talk of the meeting was from Dr. Denis Buxton (National Heart Lung and Blood Institute, NIH) on the use of nanotechnology in the diagnosis of DBA and in developing novel therapeutics.

The conference was a terrific venue for learning about the latest developments in DBA-related research.

The Daniella Marie Arturi Foundation and the DBA Foundation co-sponsored the Sixth Annual DBA International Conference April 2005 in New York City. The focus of this meeting was clinical care consensus with the goal of publishing agreed upon treatment protocols internationally for DBA. Italy, France, England, Sweden, Germany, Canada and North America were among the countries to be represented. Additionally Dr. Howard Zucker, Assistant Deputy Secretary for Health and Human Services was scheduled to speak at the meeting this year.

The next issue of the DBA Foundation newsletter will cover details of this meeting.

NOTES ABOUT THE DANIELLA MARIA ARTURI FOUNDATION

The Daniella Maria Arturi Foundation (DMAF) was established shortly after the death of Daniella Maria Arturi in 1996 by Daniella's parents, Manny and Marie Arturi. The primary purpose of the Foundation is to assist the research effort for Diamond Blackfan Anemia (DBA).

The primary focus of DMAF is scientific. The Foundation supports international medical conferences as well as domestic efforts. The DMAF has awarded research grants in the areas of gene discovery, fruit fly experimentation, registry efforts to track patients (gathering

and analyzing data), development of cell lines and ribosomal protein function studies. The Foundation is also pursuing the concept of an international Data Registry.

The DMAF has been involved in the lobbying for additional funding sources for DBA, including support from the National Heart Lung Blood Institute. The Foundation has been very pleased to witness the increased attention DBA is receiving at the NIH. In addition, DMAF recently received four mentions in the Congressional Appropriations Document this year. One of them included language directing a grant towards a Clinical Care Center at Schneider Children's Hospital in New Hyde Park, NY, which will be run by Dr. Jeffrey Lipton, MD, PhD and Dr. Adrianna Vlachos, MD.

The International Consensus Conference provides a forum for discussion exclusiviely on Diamond Blackfan Anemia between doctors from around the globe. This forum did not exist prior to the Foundation's efforts. There are discussions regarding scientific experiments, genetic discovery, animal model experiments, future directions to pursue and collaborations in new treatment protocols. Additionally, for the last two years, the Foundation has been able to offer continuing medical education units through Beam Institute, which is a testament to the educational benefits it provides.

The Daniella Maria Arturi Foundation exists for the SOLE purpose of finding better ways to treat and ultimately, to cure, Diamond Blackfan Anemia.

DBA Registry Grant Imperative to Success of Research

By Adrianna Vlachos, MD
Pediatric Hematology/Oncology and Stem Cell Transplantation
Schneider Children's Hospital, New Hyde Park, NY

The National Heart Lung and Blood Institute of the National Institutes of Health has recently awarded an investigator initiated RO1 research grant to Drs. Jeffrey Lipton and Adrianna Vlachos for $1,000,000 over the next 5 years to support the Diamond Blackfan Anemia Registry (DBAR).

Diamond Blackfan Anemia is an extremely rare disorder affecting approximately 1000 individuals in North America. Since no single center follows sufficient numbers of well-characterized patients for meaningful clinical and laboratory investigations, the Diamond Blackfan Anemia Registry of North America was established by Drs. Vlachos and Lipton in 1993 to collect accurate clinical and demographic data on DBA patients and their families. Start up funding for the DBAR was provided exclusively by the Diamond Blackfan Anemia Foundation, Inc.

The purpose of the Registry is to expand the knowledge of DBA in order to help facilitate research into the biology and characterization of the disease, to better match patients with research studies, and to serve as a resource to DBA patients and their doctors to guide diagnostic, therapeutic and reproductive decisions.

Some important DBAR research projects are described below:

1. Gene Discovery
The DBAR is collaborating with Drs. Hanna Gazda and Colin Sieff from the Dana-Farber Cancer Institute in trying to identify the second DBA gene. The Registry is particularly interested in all multiplex DBA families (those with more than one affected individual) who are willing to participate in this study.

2. Congenital Anomalies and DBA
In collaboration with Dr. Karen W. Gripp, a clinical geneticist specializing in craniofacial abnormalities at the Al Dupont Hospital for Children, DE, the DBAR is currently investigating the association between Diamond Blackfan Anemia and cleft palate. Preliminary studies have shown that there is a higher incidence of cleft palate among DBA patients than is found in the general population (6.7% vs. 0.5%). We hypothesize that DBA patients with abnormalities of the face, head and palate may represent a genetically distinct subgroup of DBA patients. In order to optimally study this association, we are inviting any DBA patient with a cleft palate to contact the DBAR for participation in this study.

3. Cancer Predisposition and DBA
The DBAR is interested in accurately assessing the risk of cancer in DBA patients. Any history of cancer in a family affected with DBA should be reported to the Registry.

4. Stem Cell Transplant Outcomes
The DBAR is collecting data on stem cell transplants done for DBA. We would like to obtain medical information on any DBA patient who has undergone transplantation to accurately assess the outcomes/risks of stem cell transplant in these patients.

Since this is such a rare disorder, it is only through the participation of every individual with DBA and their families that we can have any meaningful insights as to the causes and best treatments for DBA.

Please contact the DBAR coordinator, Eva Atsidaftos, to enroll or check on the status of your registration.
Call toll-free 888-884-DBAR (3227) or e-mail eatsidaf@lij.edu
You can also visit our website at www.dbar.org.

Thank You to Our Families and Friends

The Diamond Blackfan Anemia Foundation, Inc. (DBAF)'s mission statement reads, "Our mission is to collectively and actively generate funds for the charitable and scientific purpose of furthering, by clinical study, laboratory research, publication and teaching the knowledge of the disorder known as Diamond Blackfan Anemia (DBA). Our intentions are to share this knowledge, to inform, to lend support, and to communicate with all families of DBA patients."

In order to fund approved research projects, the DBAF relies on our families and friends for contributions and fund raising. We are grateful for the commitment and hard work of the families and friends of the following DBA patients. Their efforts have provided the DBAF the opportunity to continue funding worthwhile research projects.

We strongly encourage ALL our families to get involved. We need your help to continue to support ongoing research. If you would like to make a contribution, please send your tax-deductible donation to: Diamond Blackfan Anemia Foundation, Inc. PO Box 1092 West Seneca, NY 14224. If you would like to organize a fundraiser, or would like more information, please contact Dawn Baumgardner at 716-674-2818. THANK YOU!!!

Brandi Adams
Kevin Ballina
Justin and Kyle Baumgardner
Lizzie Bell
Cassondra Belotti
James Bohuski
Paula and Sarah Browning
Kate Burnette
Sean Cadden
Shannon Childs
Gail Coughlin
William Fair
Brianne Fitzmaurice
Tori Flavahan
Kevin Gately
Shayna Goldrich
Kathleen Grace Green
Kristine Gunderson
Alexandria Hartmann
Natalie Hianik
Cameron Lanore
Paige Mauch
Nancy McSweeney
Kylie Monica
Elisabeth Paye
John Paul Quintero
Kyle Rashford
Coleson Shaw
Carson Souza
Ryan Spring
Katie Trebing
Deana Valentino
Jeffrey Vink
Christopher and Matthew Vroman
Andreas Wagner
Dan Wagner
Sean Ware
Atleigh Whitman
Jorrit Zeldenthuis

In Loving Memory of:
Katie James
Brittany Seitz
Keir Zangrando

National Blood/Bone Marrow Drive

Kathy Bell, Mom to DBA patient Lizzie Bell and Co-Chair of the John P. Bell Family Foundation, is coordinating a National Blood/Bone Marrow Drive to be held by the American Red Cross in honor of Diamond Blackfan Anemia on May 21, 2005 (the national day of recognition for the American Red Cross).

The John P. Bell Foundation exists to support the caregiver or grass roots individual who must continuously serve others… by forgetting self. The foundation is also dedicated to funding programs and projects that embrace hope when the unusual, the out of the ordinary or the unexpected happen in life.

For more information on this drive, its locations and the goals it hopes to attain please contact Kathy Flores Bell at Azbells@aol.com.

DBA Wrist Bands are Coming

Beginning in mid May, silicone wristbands in the fashion of the Lance Armstrong "Live Strong" yellow bands, will be available to those interested. They are red and debossed to say "Diamond Blackfan Anemia". Wristbands are available in Adult size (8 1/4 inch) and Youth size (7 1/4 inch).

Bands will cost $2.00 each with a minimum order of 10 plus $5.00 for United States shipping. Shipping is available out of the United States by contacting the email address below. Please contact Twila Edwards at tke73@hotmail.com and put "DBA" in the subject line.

All proceeds from this fundraiser/awareness project will go to the Diamond Blackfan Anemia Foundation.

When Stealing is OK

By Bob Gunderson

Ok, I admit it. I am a thief. I steal from others, and on a regular basis. And I have been successfully stealing for several years now. But I don't think I'll be in jeopardy of jail time for this theft. Nor even more important, I am not in fear of upsetting God with this one. In fact, this habit of theft has so enriched my life, I am going to urge all of you to try it occasionally.

Encouragement. This is my weakness. I like to be encouraged. When faced with life's biggest battles, just let me steal some encouragement from somewhere, and I will make it through. Now, I am fully aware that on my own I am fairly incompetent and under-motivated. Some would apply the word "slack". But I put to you, it is not slackness but the sure knowledge that on my own I am over-whelmed. But I discovered something a few years ago that changed my life and led me into a life of theft. I was invited to a fundraiser for DBA in Atlanta, GA. Actually, it's Acworth, Ga, but for anyone not in Georgia, let's just call it Atlanta. The Moonbeams For Diamond Gala and Silent Auction, and the KVO Golf Tournament is hosted by Tim and Kathi Vroman and their dear friends, the Musials. And WOW!! What a fundraiser it is! A weekend of fun and laughter and joy. And when I attended this fundraiser, I discovered that I could easily make away with all the encouragement I could carry at one time – at least enough to get me through part of the next year. And the hosts of this fundraiser were so nice and generous, they seemed to not care that I was carrying away so much encourage-ment. They must be naïve, because they actually invited me back the next year! And I stole more encouragement. In fact, I finally talked my wife Lisa into going, so she could steal some too! I have carried friends to Georgia to steal. I even took my best friend Ken Thrasher, who is a Methodist preacher. And he stole some too.

Then, as I became addicted to this encouragement I started looking for other places to steal some. I found a fundraiser in Virginia called Swampfest. This was a little different from the Vromans' weekend, but hey, it involved Jello Shooters and a live band, and more good people, fun and laughter, more encouragement that I could spirit away when the day was done. Then, right in my own backyard, the Diamond Blackfan Fishing tournament. Wow! I am really hooked (get it? – fishing , hooked, ha ha) on stealing encouragement.

I am telling you people, you gotta try this! When your day is dark, when you are overwhelmed by the long term prognosis, when you are just not sure you can make it through another round of doctors and needles and wondering if your child will be well or sick, when you feel scared or alone or helpless, you need encouragement. If you stashed some away at a fundraiser, you just use some up to chase away the blues and the fear. Take some of the encourage-ment that you are not alone in this fight and that there are hundreds of good, good people out there fighting to solve the very problem that you see as insurmountable. And fundraising encouragement is some of the best you can find. When you see hundreds of strangers come together to donate thousands of dollars to an obscure (albeit important) cause, you get such a dose of encouragement as you hardly ever see. So please, this is one bad habit I encourage you to take up. Share in the joy and peace and find a fundraiser for DBA to attend so you can steal some encouragement.

Peace,
Bob

CAMP SUNSHINE: FUN, SUN, AND SHARING

Set among the tall pine trees and a stone's throw from a large, beautiful lake, it's always a great experience going back to Camp Sunshine near Portland, Maine. Sunshine, volleyball, swimming, art, water sports, wall climbing, blooper games, and now even air conditioning were among the many amenities offered. Throw in great counselors, bonfires, with smores, lots of food, old friends, new friends and medical presentations and you have the setting for a week with DBA families from North America, Holland, and England.

The Lanore family (Michigan) and friend Shannon from Massachusetts get into the spirit of the costume party.

The volunteers and "friends" all sing a tearful version of "That's What Friends are For" as they bid farewell to the families.

Barriers needed by kids and adults in everyday life were easily shed. Campers were able to talk freely about their illness, show off and compare their chelating pumps and supplies, and cry more easily. Curfews were broken as parents gathered outside their rooms to socialize and talk about DBA as well as life in general. Some kids experienced their first sleepover, slumber party, and campout all in the same week!

Speakers attending were Drs Jeffrey Lipton, Adrianna Viachos, Blanche Alter, Hanna Gazda, and Steve Ellis. These doctors are all very committed to DBA research and spent a lot of time with DBA families both in group settings and individually. The families all appreciated these doctors taking the time out of their very hectic schedules to spend a few days at camp with them.

Mark LaPierre (Maine) seen with family friends, reunites with Drs Jeffery Lipton and Blanche Alter at Camp Sunshine.

The moms are treated to a serenade by the dads on Adult's Night Out.

Some adults found the psychosocial group discussions to be emotional, yet necessary and felt more discussions would be beneficial. Attendees were fortunate to have the experience and wisdom of Nancy Cincotta, MSW, to lead them through the various issues addressed.

Marci Mauch (Kansas) and Tara Flahavan (Minesota) take a minute to pose for a photo.

Eran Jenny-Schmidt (Maryland) and Tori Flahavan (Minnesota) hang out at Camp Sunshine.

For many, the most difficult part of camp was missing two children who had undergone bone marrow transplants since the previous camp and passed away following complications from their transplants. Both had HLA compatible sibling donors. Several mentioned that they were almost not able to come because of how difficult emotionally it was to deal with the loss and week without them.

Encouragement was found from two teenagers who had been at camp in previous years and had also undergone HLA compatible sibling donor transplants. These two looked and felt great and were willing to share their experiences with all who sought information from them. One of these teens passed up his chance to make a wish with the Make-A-Wish Foundation because his wish has already come true.

The Childs family from Massachusetts poses for a family portrait.

Two families brought great videos of their DBA children that they used to promote blood drives and blood donations in their areas. One of the videos is even on television. Both were excellent, informative and at times sad seeing and knowing what DBA children go through daily.

Another teen, also a camp regular, is now in remission after 15 years of transfusions! That was very exciting news and everyone was happy for him! We're all hoping the same for our own children.

We're all looking forward to going back to Camp Sunshine in 2006 and hope more new familes are able to join us for this fun, informative, and very busy week!

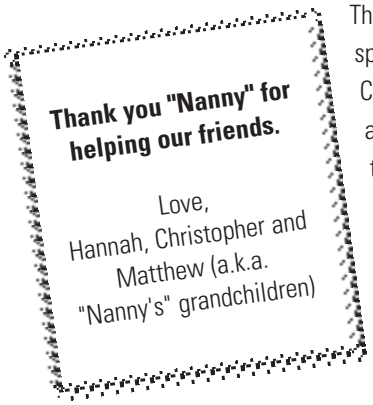
A tender moment as children and their families launch their wish boats…and the couselors look on from the bridge.

Community Weaves Love and Hope into Their Quilt

Dottie Kilcoyne looks forward to "quilt night" in her small town of Clinton, Massachusett every month. And why not? Here is a group of women who share their passion for creating masterpieces out of scraps of cloth. And, while they work together, they share the hopes, dreams, and heartaches of their own lives as well as those of their children and grandchildren. It is this small group of quiet and unknown "artists" that decided to help a very small group of children with an unknown and rare disease.

See, what these women know is that two of Dottie's six treasured grandchildren have a rare blood disease that most have never heard of – Diamond Blackfan Anemia (DBA). Dottie told the women about her family attending Camp Sunshine this summer and what it meant for them to be among other DBA families.

Every other year, 40 families from around the world find one another and meet in a beautiful and unassuming place in Casco Maine -- Camp Sunshine. For one amazing week DBA children can feel "normal". They swim, boat, run and play with other children just like them – kids who maybe can't run that fast, throw that far or climb that high. Their siblings can feel "normal" too – here are other kids who know the stress and strain of having a brother or sister who's just a bit different. But it's for the parents that Camp Sunshine is the most wonderful experience. Doctors from around the world come to Camp Sunshine to present parents with the latest medical advances in this rare disease. They eat meals with the parents to listen and answer questions. They see the children for whom they are working for a cure. And they give the parents hope…



This year, Dottie's quilting guild decided to help. Two beautiful quilts were raffled to raise money to sponsor two Diamond Blackfan Anemia families to Camp Sunshine – families who might not get to Camp Sunshine without their help. The quilts were put on display at the local hospital, churches and town fair. All the women took tickets and sold chances to buy these treasures to everyone they met. And, because of their help, two families can be sponsored to the next Camp Sunshine.

The guild developed the quilts unique pattern with this unknown group of children in mind. And most of these women don't know much about DBA either. But they know that their good friend Dottie who doesn't say much about this disease, quietly suffers for her grandchildren and prays daily for a cure.

Rockin' and Rollin' in San Diego

Jason Martin "huffs and puffs" as he runs a 26 mile marathon in San Diego, California. Jason ran the marathon on the weekend of June 5th, 2004 and raised over $2,000 for DBA research. Ryan Spring, Jason's cousin, has DBA. We are proud of Jason's efforts.

Thank You, Jason!

Getting Out the Word

By Marisol Quintero

"Congratulations, you have a baby boy"…was all I heard and thereafter, I heard the worried sighs and saw the expressions of the staff. Four years ago, on March 9th, 2001, my son John Paul Quintero, was born. We thought we had had a healthy almost 9 lb baby boy, until they informed us that he would be getting transferred via ambulance to another hospital for transfusions. We brought him home 12 days later and we thought that we had put the nightmare behind us. Little did we know that was just the beginning. On May 2nd, 2001, at only a month and a half, John Paul was diagnosed w/ Diamond Blackfan Anemia. We tried the steroids, the cyclosporin, the cellcept, the reglan, and during this year and a half, he was still getting transfused. So, we decided to take him off of everything and just do the transfusions alone. Four years later and 83 transfusions, we still have our ups and our downs But I do not give up hope. That is why, I have become a huge advocate for DBA awareness and Blood Donation awareness as well. Not only have we been featured on news channels, but we were also aired on a psa for the American Red Cross that just this January, was aired nationwide.

We too have had our share of fundraisers, from Firemen car washes, art gallery auctions, to our two most recent which was our very first big event called "Drops of Hope" and a bakery fundraiser event. There are many kind people out there who are willing to go above and beyond for your cause. We just need to be more vocal about DBA and not be afraid to speak. This owner of a bakery in a nearby neighborhood, called Viva Bakery, heard about DBA through me. What did he do? He decided to focus on one day, make a bread in honor of John Paul, sell that bread for $3.00 and give that money to the DBA foundation. Well, it happened. On November 19th, 2004, Mr. Adrian Ezrre of Viva Bakery worked 48 hrs to make this event happen. We raised a little over $1000.00 that day by selling a bread in the shape of a horseshoe (John Paul loves horses) and because it is the sign of good luck, we decided to name the event, "A Bite of Goodluck". It brought so much awareness, they featured this on an exclusive air time on the spanish channel Univision and it brought many people from near and far, to help us out with the cause. One man said he would match the amount up to 500.00 and that is what he did. Yes, fundraisers can be a lot of work, but at the end, they are so well worth it. It only brings us that much closer to finding a cure.

We don't know what the future holds for John Paul, nor for many of the DBA patients. But I do know one thing…there will come a day when they'll all know about DBA.

Do You Have A Story to Share?

Do you have a personal story of encouragement or inspiration that may offer some hope to other DBA families? If so, we'd love to hear it!

The DBA Newsletter is intended to provide the medical community and DBA families and friends with updated information on medical advances and research impacting Diamond Blackfan Anemia. However, for some families and friends, it also may be the only opportunity they have to affiliate with other DBA families and learn more about coping on a day to day basis.

If you or your child has a story to share, please contact Kathi Vroman at kavroman@aol.com.

DBA Foundation Website Provides Assistance to Fundrasiers

Did you know that the DBA Foundation owns the rights to an inspirational song written specifically for DBA fundraisers? The Atlanta Young Singers of Callanwolde wrote and performed the son "Miracle to Fly" 2 years ago at a DBA fundraiser in Georgia.

Additionally, there is a multi-media presentation available to anyone who is having an information session or other event involving Diamond Blackfan Anemia. This program was written and developed by Bill and Cheryl Musial of Mopdog, Inc. It can be found on the Diamond Blackfan Anemia website - www.dbafoundation.org/fundraising.html - and can be downloaded to your computer for your review.

For more information on using these to assist in your fundraising or DBA awareness efforts please email Kathi Vroman at kavroman@aol.com.

Miracle to Fly

Intro: (What is a Miracle? Faith…Hope…Love)
Oo…Oo
1st Verse: (Faith)
Beauty waits present in this unseemly form;
I rest. I wait.
Nestled where the clouds spend the night.
You, with unknowing eyes shall see,
Shall see this miraculous design.
Chorus:
And I shall Fly born in air aloft on painted wing
Wings, once held close, release.
Moved from darkness,
Darkness into light.
I await a miracle to fly.
2nd Verse: (Hope)
Miracle, Patient in the land of in between.
I rest. I wait.
Journey to a greater call.
Here, in the dark unseen I move.
Striving, yearning, turning to break free!
Chorus:
And I shall fly, born in air aloft on painted wing.
Wings, once held close, release.
Moved from darkness,
Darkness into light.
I await a miracle to fly.
Ah…Ah..Oo..Oo..
3rd verse: (Love)
Life renewed. Vibrant in the air beyond.
I see. I feel. Lifeblood flows drowsy from sleep.
Stretching, unfurling wings are filled.
Illumined as prisms in the sun!
Chorus:
And I shall fly born in air aloft on painted wing.
Wings, once held close, release.
Moved from darkness,
Darkness into light
I await a miracle to fly.
Fly away…fly away…ah (What is a Miracle? Faith, Hope & Love)
Solo:
Miracles are caterpillars when butterflies have flown.

I AM

By: Christopher Vroman
Age 10, Transfusion and Desferal dependent

I am different and special at the same time.
I wonder how people began without parents.
I hear the sound of imaginary people calling my name.
I see things getting darker by the second.
I want nothing more than my family.
I am different and special at the same time.

I pretend that I am a super hero.
I feel the coldness of a ghost.
I touch the grip of rubber.
I worry that no one likes me.
I cry that I am different.
I am different and special at the same time.

I understand that some things are impossible.
I say that Santa is real.
I dream that I have a machine to hover through obstacles.
I try to get good grades.
I hope my family will never argue again.
I am different and special at the same time.

DBA RESEARCH ARTICLES 2004

Abstracts of many of these articles can be read online at: http://www.ncbi.nlm.nih.gov./entrez/query.fcgi

- **Development of cellular models for ribosomal protein S19 (RPS19)-deficient diamond-blackfan anemia using inducible expression of siRNA against RPS19.**
  Miyake K, Flygare J, Kiefer T, Utsugiawa T, Richter J, Ma Z, Wiznerowicz, Trono D, Karlsson S
  Mol Ther. 2005 Apr.11(4):627-37
  PMID: 15771965 [PubMed – in progress]

- **Central venous catheter thromosis as a cause of SVC obstruction and cardiac tamponade in a patient with Diamond-Blackfan anemia and iron overload.**
  Norris ML, Klaassen R
  Pediatr Blood Cancer. 2005 Mar 14: [Epub ahead of print]
  PMID: 15768384 [PubMed – as supplied by publisher]

- **An RNA interference model of RPS19 deficiency in Diamond-Blackfan Anemia recapitulates defective hematopoieses and rescue by dexamethasone: identification of dexamethasone responsive genes by microarray.**
  Ebert BL, LeeMM, Pretz JL, Subramanian A, Mak R, Golub TR, Sieff CA
  Blood. 2005 Mar 8: [Epub ahead of print]
  PMID: 15755903 [PubMed – as supplied by publisher]

- **Pediatric red cell disorders and pure red cell aplasia.**
  Perkins SL
  Am J Clin Pathol. 2004 Dec: 122 Suppl:S70-86. Review.
  PMID: 15690644 [PubMed – indexed for MEDLINE]

- **Gene Symbol: RPS19. Disease: Diamond-Blackfan anemia.**
  Goncalves P, Pereira JC, Kidd AM, Ribeiro ML
  Hum Genet. 2004 Nov:115(6):534. No abstract available.
  PMID: 15678589 [PubMed – indexed for MEDLINE]

- **Deficiency of ribosomal protein S19 in CD34+ cells generated by siRNA blocks erythroid development and mimics defects seen in Diamond-Blackfan anemia.**
  Flygare J, Kiefer T, Miyake K, Utsugisawa T, Hamaguchi I, Da Costa L, Richter J, Davev EJ, Matsson H, Dahl N, Wiznerowicz M, Trono D, Karlsson S
  Blood: 2004 Dec 30:[Epub ahead of print]
  PMID: 15626736 [PubMed – as supplied by publisher]

- **Postmortem diagnosis of Diamond-Blackfan anemia.**
  Beauchamp-Nicoud A, Da Costa L, Proust A, Rince P, Saker S, Tchernia G
  J Pediatr Hematol Oncol. 2004 Dec:26(12):847-8.
  PMID:15591911 [PubMed – indexed for MEDLINE]

- **Marrow failure.**
  Bagby GC, Lipton JM, Sloand EM, Schiffer CA
  Hematology (Am Soc Hematol Educ Program). 2004;:318-36
  PMID: 15561690 [PubMed – in progress]

- **Elevated thrombopoietin levels and alterations in the sequence of its receptor, c-Mpl. In patients with Diamond-Blackfan anemia.**
  Pospisilova D, Cmejlova J, Slavik L, Cmejla R
  Haematologica. 2004 Nov:89(11): 1391-2
  PMID:15531462 [PubMed – in progress]

- **Diamond-Blackfan anemia responding to valproic acis.**
  Jabr FI, Aoun E, Azar C, Taher A
  Blood. 2004 Nov 15;104(10):3415. No abstract available.
  PMID:15525842 [PubMed – indexed for MEDLINE]

- **Nonsence-medicated and nonstop decay of ribosomal protein S19 mRNA in Diamond-Blackfan anemia.**
  Chatr-Aryamontri A, Angelini M, Garelli E, Tchernia G, Ramenghi U, Dianzani I, Loreni F
  Hum Mutat. 2004 Dec; 24(6):526-33
  PMID: 15523650 [PubMed – in progress]

- **Successful transplantation of CD34+ selected peripheral blood stem from an unrelated donor in an adult patient with Diamond-Blackfan anemia and secondary hemochromatosis.**
  Berndt A, Helwig A, Ehninger G, Bornhauser M
  Bone Marrow Transplant. 2005 Jan;35(1):99-100. No abstract available.
  PMID: 15516941 [PubMed – in progress]

- **RNA and protein evidence for haplo-insufficiency in Diamond-Blackfan anemia patients with RPSI mutations.**
  Gaza HT, Zhong R, Long L, Miewiadomska E, Lipton JM, Ploszynska A, zaucha JM, Vlachos A, Atsidaftos E, Viskochil DH, Niemever CM, Meerpohl JJ, Rokicka-Milewska R, Pospisilova D, Wiktor-Jedrzejczak W, Nathan DG, Beggs AH, Sieff CA
  Br J Haematol. 2004 Oct;127(1):105-13.
  PMID: 15384984 {PubMed – indexed for MEDLINE]

- **Growth hormone therapy for short stature in Diamond-Blackfan anemia.**
  Scott EG, Haider A, hord J
  Pediatr Blood Cancer. 2004 Oct;43(5):542-4.
  PMID: 15382270 [PubMed – indexed for MEDLINE]

- **Couple allowed to select an embryo to save sibling.**
  Dyer C
  BMJ. 2004 Sep 11;329(7466):592. No abstract available.
  PMID: 15361442 {PubMed – indexed for MEDLINE]

- **Two-phase culture in Diamond Blackfan anemia: localization of erythroid defect.**
  Ohene-Abuakwa Y, Orfali KA, Marius C, Ball SE
  Blood. 2005 Jan 15;105(2):838-46. Epub 2004 Jul 6.
  PMID: 15238419 [PubMed – indexed for MEDLINE]

- **Successful nonmyeloablative bone marrow transplantation in a corticosteroid-resistant infant with Diamond-Blackfan anemia.**
  Ostronoff M, Florencio R, Campos G, Arruda S, Matias C, Florencio M, Domingues M, Maior AP, Sucupire A, Calixto R, Tagliari C, Matias K
  Bone Marrow Transplant. 2004 Aug;34(4):371-2. No abstract available.
  PMID: 15220956 [PubMed – in progress]

- **Preimplantation HLA testing.**
  Verlinsky Y, Rechitsky S, Sharapova T, Morris R, Taranissi M, Kuliev A
  JAMA. 2004 May 5;291(17):2079-85.
  PMID: 15126435 {PubMed – indexed for MEDLINE]

- **Targeted disruption of the ribosomal protein S19 gene is lethal prior to implantation.**
  Matsson H, Davey EJ, Draptchinskaia N, Hamaguchi I, Ooka A, Leveen P, Forsberg E, Karlsson S, Dahl N
  Mol Cell Biol. 2004 May;24(9):4032-7.
  PMID:15082795 [PubMed – indexed for MEDLINE]

- **Molecular basis of Diamond-Blackfan anemia: new findings from the Italian registry and a review of the literature.**
  Campagnoli MF, Garelli E, Quarello P, Carando A, Varotto S, Nobili B, Longoni D, Pecile V, Zecca M, Dufour C, Ramenghi U, Dianzan I
  Haematologica. 2004 Apr;89(4):480-9.
  PMID: 15075082 [PubMed – in progress]

DBA Foundation Wishlist

The mission statement of Diamond Blackfan Anemia Foundation is that it exists to "collectively and actively generate funds for the charitable and scientific purpose of furthering, by clinical study, laboratory research, publication and teaching, the knowledge… Diamond Blackfan Anemia". Expenses for administrative items needed to keep the Foundation running are paid for by donations from families and friends. If you or your business can help out with any items on our "wishlist" please contact Dawn at dbafoundation@juno.com

Wishlist:

- Stamps
- Phone cards
- Copy paper
- Phone with Answering Machine
- Laptop Computer
- Small Copy Machine
- New computer and printer

Diamond Blackfan Anemia Foundation, Inc.
20 Tracy Lynn Lane
West Seneca, NY 14224
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Design donated by:

mopdog.com • 770.874.4983

Printing donated by:

770-218-2777 • 770-218-2720 (fax) • Kennesaw, GA

Diamond Blackfan Anemia Registry

For those of you needing to contact or mail medical records to the Diamond Blackfan Anemia Registry (DBAR), please use the following information.

MAILING ADDRESS:

Diamond Blackfan Anemia Registry
c/o Dr. Adrianna Vlachos
Schneider Children's Hospital
Division of Pediatric Hematology/Oncology and Stem Cell Transplantation
269-01 76th Avenue
New Hyde Park, NY 11040

TOLL-FREE PHONE NUMBER:

1-888-884-DBAR

EMAIL ADDRESS:

Dr. Vlachos can also be reached by e-mail at:
avlachos@lij.edu.