



**DIAMOND BLACKFAN ANEMIA
FOUNDATION, INC.**

Diamond Blackfan Anemia Foundation, Inc.
20 Tracy Lynn Lane
West Seneca, NY 14224
www.dbafoundation.org

Spring 2010

Sponsored by:



**DIAMOND BLACKFAN ANEMIA
FOUNDATION, INC.**

DBA NEWSLETTER

Diamond Blackfan Anemia

for patients...for families...for research

The DBA Foundation, WE are YOU!

The DBA Foundation, Inc. (DBAF) is a nonprofit organization made up of family and friends of those affected by Diamond Blackfan Anemia. In short, WE are YOU. The DBAF's 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. Our intent is to educate, inform, empower, support and to connect the entire DBA community." We have listened to your suggestions regarding the DBA Newsletter and appreciate your feedback. Our hope is to publish a newsletter that will more closely resemble our mission.

We strive to make this newsletter a beneficial publication for all affected, thus your ideas and participation are not just welcomed, but they are needed. We are committed to publishing a document that not only informs but supports and even challenges its readers. Please read with the acceptance that some of the data may not be applicable to you at all times, but know that we are attempting to reach the diverse population affected by DBA.

The newsletter will be mailed out only twice a year, so in the meantime please visit our website, www.dbafoundation.org, that was launched in 2009 to provide the public with accurate and timely information. Our website is updated frequently and is an excellent resource for information and support. Visit our website for information regarding DBA and links to connect with DBA families from around the world on Facebook and web-based support groups. You can also follow us on Twitter!

We sincerely thank you for your support and we look forward to your continued partnership as we strive to make a difference in the lives of all of those affected by DBA.

A Tale of Two Heroes

Gaining perspective.

When I put aside the memories that cloud my internal rear-view mirror, I can define that "a-ha" moment to the day my young family and I stepped inside the office of Dr. Lipton and Dr. Vlachos in New York. We arrived on the Island a prisoner of our fears and left feeling fortunate that we weren't dealt a tougher hand. We had been given the knowledge needed to be able to secure appropriate care for our son. Once we allowed ourselves the time to accept our new lifestyle and the worries that DBA had delivered, DBA shifted from the center of our existence to just a part of our lives.

Continued on Page 3



Camp Sunshine applications are NOW available at www.campsunshine.org!

The DBA Foundation invites all of our families to apply to be a part of this life-changing experience! Space is limited, so the DBAF urges you to send in your application soon.

Camp Sunshine will host our families during the week of **July 11-16, 2010**. Please visit www.dbafoundation.org or call Dawn Baumgardner at 716.674.2818 for more information. Applications can be downloaded from www.campsunshine.org, or requested by calling Camp at 207.655.3800. Completed applications should be returned promptly to Camp Sunshine (not to Dawn). Please read the Camp Sunshine article in this newsletter!

The DBAF is proud to continue our mission of supporting DBA patients, families and research. We are grateful for the support and involvement of many of our families, and we encourage all DBA families to partner with us as we strive to reach our goals.

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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 / Eva Atsidaftos, MA
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

E-MAIL ADDRESS:

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

Eva Atsidaftos can be reached by e-mail at:
eatsidaf@lij.edu

WEB ADDRESS:

www.dbar.org



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We would love to hear from you!

DIAMOND BLACKFAN ANEMIA FOUNDATION

1 (716) 674-2818

DBA NURSE HOTLINE

1 (877) DBA-NURSE

Camp Sunshine



About this time of year when the dates are announced, my husband and I actually remember how the anticipation of our first trip to Lake Sebago

made us feel. Our every thought was consumed by the promise of finally meeting other families who also had their very existence altered by the very same three letters as we had. Those families who had once stood in our shoes were now ready and willing to help us navigate through these rough waters to calmer waves. Camp Sunshine was the antidote to our sea sickness. In less than one week's time, strangers had become family and their open arms, an anchor.

For the benefit of us all, the DBA Foundation has ensured that Camp is so much more than just a chance to meet other families. In years past it has been arranged for various experts to visit the daily sessions, presenting the most up-to-date information related to DBA as well as providing us with the answers to many of the questions that we need in order to get the best care for our loved ones. Our trip to Camp in 2006 featured representation from the NIH, an expert on iron overload, various researchers studying DBA as well as an update from Dr. Jeffrey Lipton and Dr. Adrianna Vlachos of the North American DBA Registry. The highlight of Camp for our family was the chance to arrange a short session to answer individualized patient care questions with Dr. Lipton and Dr. Vlachos, an opportunity they extended to each family in attendance. The schedule also left time to bond with other families which afforded me the chance to learn about the real-life ups and downs of living...and thriving with DBA.

Attendance at this amazing Camp is offered to our families free of charge. However, participating families are required to provide their own transportation to/from Camp or the airport in Portland, ME. There is 24-hour medical support available. The week is full of fun and education and is a wonderful opportunity to make new friends and reconnect with old ones. This year, Camp Sunshine will host our families during the week of July 11-16, 2010. Please visit www.dbafoundation.org or call Dawn Baumgardner at 716.674.2818



Our DBA kids at Camp Sunshine 2006.

for more information. Applications can be downloaded from www.campsunshine.org, and completed applications should be returned to Camp (not to Dawn). The DBA Foundation invites all of our families to apply to be a part of this life-changing experience!

There has been a lot of interest in Camp this year, so the DBAF urges you to send in your application soon.

Camp Sunshine 2008



Photo collages compliments of Heather Monica.

DBA Awareness

Facebook, wristbands, media coverage... Why does visibility for DBA matter?

Those of us receiving this newsletter are probably not the reason why spreading the word about the DBA Foundation is important, but it's those families out there who are fighting this battle without support or current data on diagnosis and treatment that need to know that we exist. When we are able to connect with these families, we can direct them to the DBA Registry, to knowledgeable doctors, to support groups and to a compassionate ear. There are approximately 25 new DBA cases diagnosed each year, and those parents need information. There are also people out there who have dealt with their DBA for years and never knew of or contacted the Registry. This means that their health data has not been reported, therefore important pieces of the DBA puzzle are missing. Please help us to help all our families. If you are aware of any families that are not registered with the DBAF, please direct them to our website www.dbafoundation.org or call Dawn Baumgardner at 716.674.2818. **THANK YOU!**



DBA wristbands are a great way to show support and raise awareness for DBA. Many have made the wristbands available at fundraising events, asking friends and family to support the DBAF by wearing the wristband and making a *minimum* donation of \$2.00. Please contact Twila at twilak@cox.net to arrange your purchase.

A Tale of Two Heroes *Continued from Page 1*

For us all, time passes and life moves on. Most days are good in spite of DBA, but we all know how a single set of labs can return us to the state of mind in which the medical routine seems relentless and exhausting—where sometimes just surviving is all we have the energy for and inspiration seems out of reach. Then, just when you need it the most, a true hero appears (and sometimes even two) to scoop you up and fly you high above the storm to give you the brand new perspective you have been desperately waiting for... a life-altering invitation to change the way you view the world.

A different perspective.

Meet Casey Leonard. To Casey, DBA isn't all-too-regular clinic appointments or nightly needles and chelation. To Casey, DBA is a thief. Casey's story started much like the rest of ours but within three months time her daughter's battle was lost, and the little life she had made was stolen before her very eyes. Beautiful Lakshmi was gone and Casey was left with more pain than a parent should ever have to bear. Months without answers were torturous and finally the results of the autopsy were in. Though no gene was identified, Lakshmi's marrow resembled those of our own loved ones', and Diamond Blackfan Anemia became the diagnosis Casey was left to blame. When the anger had dwindled and the sadness consumed her, running became cathartic and was all that was getting her out of bed in the morning. The distraction that began as 5K runs had evolved to joining the Team in Training and her first marathon; what was her therapy had become her mission.

For Lakshmi's story to live on, Casey felt compelled to uphold her daughter's memory in a way that would also raise money to fund the research meant to unravel the mysterious disease that had forever changed her world. For Casey, funding research keeps a promise alive that someday, when her Lakshmi's gene is finally identified and the time and circumstances are right, that she may be ready to become a mother again.

"I run for Lakshmi"

These four words may have started as Casey's swansong of sorts, but it seems that so many more have been inspired by her strength and have adopted these sentiments as their own. On May 1st and 2nd, eleven of Casey's family and friends will join her in the first overnight relay between Boston and Provincetown— with four more teammates driving the support vans. The inconsecutive legs vary in length and difficulty and will both test its contestants and counsel them. This rigorous trek, a gesture in honor of the beloved "Lady Luck," the inspiration for Casey and her loved ones, is a race her DBA family can join as well.

To get behind Casey and support her cause—our cause—please visit and forward her website, www.teamlakshmi.com. Donations will be directed to the DBAF and go towards research and projects approved by the Board.

An outsider's perspective.

Joe Crelier's motivation seems as if it derived from the opposite side of the heart as Casey's. His less painful path to inspiration needed, craved a cause to fight for. He had signed up to be a part of the Janus Charity Challenge and, along with securing his spot at the Idaho Ironman; he

needed to choose the recipient of his fundraising efforts. For Joe, a critical component in this decision was to make a heartfelt connection. He wanted to feel that click... the peace that comes when you know you've found just what you've been looking for.



Joe Crelier and his family. Joe is training for an Ironman and raising money for DBA.

Years earlier, Joe and his wife's commitment to blood donation had been solidified by a simple "thank you" note he had received from grateful parents after a recent donation had helped their 19 month old baby. Little did he know that the vow he made on that day to become a dedicated blood donor would someday culminate into helping masses of people who are unable to make their own.

Still soul-searching for his cause, he found his interest to be piqued by the trailers for the upcoming episode of ABC's Extreme Makeover in which the Bell Family were to be the featured guests. Their daughter, he learned, required monthly blood transfusions to survive. Before 9 o'clock Mountain time on that Sunday night, the good deed he performed every 8 weeks was abstract, but after the best of reality television introduced him to Miss Lizzie Bell, who shared his appreciation for A positive blood, it became personal.

"Not only had I found a connection, I had felt the connection to go with the inspiration. I looked up Diamond Blackfan Anemia, and I was certain I had found my Ironman cause."

A man, completely unaffected by DBA, who enjoys cooking and family and sports watches an hour of television and suddenly we, the tiny DBA community struggling to fund research in search of a cure for our loved ones, are to benefit from the windfall that partnering with Joe brings. Long distance, technology-enabled, 21st Century love-at-first-sight.

"Not only will your stories be motivating me on race day, they motivate me all the time. I'm so glad to be partners with you on the journey to a cure."

As you can see, Joe is touched by the personal sentiments that our families have sent his way and is impressed by the dollars generated from our connections. Part of his goal, however, is to attract donations from others like him who are not previously aware of DBA, broadening the scope of potential donors. To aid Joe in his mission to raise \$100,000 to benefit our families, we must continue to send out the link to his website and share his story. If you would like, you are welcome to copy the following quote and link and pass it along in your attempt to spread his word, our word.

Will of Iron. Heart of Gold. A virtual stranger...a man completely unattached to DBA is training for an Ironman and while pounding the pavement/pool/peddle, nursing his blisters and icing his aches, he is also attempting to raise \$100,000 for the Diamond Blackfan Anemia Foundation. Please go to <http://januscharitychallenge.kintera.org/cda10/dbaf> to lend Joe a hand.

While Casey and Joe log hundreds of miles in the next few months preparing to achieve their heroic goals, let us, the DBA community, band together to do our part and make their missions our own. Please do not stand idle and miss out on these opportunities to further research, as the futures of our loved ones are at stake.

Ask A Nurse

In that so few physicians and nurses have much experience with DBA, a call to one's local clinic in search of advice about a vaccine or for support in getting a toddler to ingest Exjade is oftentimes fruitless. It is the Foundation's impression that many could benefit from greater access to a DBA expert, and for this reason we would like to introduce the *Ask A Nurse* section of the newsletter. We are very fortunate to have access to Ellen (1-800-DBA-NURSE), the DBA nurse at Dr. Lipton and Dr. Vlachos' office in New York, as well as Debbie, at the DBA Resource Center in Texas who have both agreed to field our DBA-related questions. If you have a question to submit for future editions please email it to jdowney@dbafoundation.org or dbafoundation@juno.com so it can be directed to the designated DBA nurse. By publishing these responses, we hope to allow many more families to benefit from the wisdom of our experienced nurses.

Dear Ellen,

I am very concerned about H1N1 and do not know if my child with DBA should get vaccinated. Would you please give me some advice on this matter to share with our local physician?

Sincerely,
A Concerned Parent

Dear DBA Community,

It cannot be stressed enough that every DBA patient has a different situation and response to illness. Please work with your hematologist, pediatrician or internist in deciding what are the best options for you and your family. These are general recommendations. The care of each patient should be individualized with his or her physician.

There have been several inquiries about H1N1 (swine flu) and DBA. We at the DBA Registry advise the patients treated here at Schneider Children's Hospital in New York to get immunized for both the H1N1 virus as well as the seasonal flu. If you have received the seasonal flu vaccine in the past with no issues, the H1N1 vaccine is being prepared in the same way and should potentially be safe for you or your child.

If you have severe, life threatening allergies, reactions or events to any of the substances in the vaccine, you should not get receive it (eggs or preservative). There is a preservative free form of the vaccine which contains no Thimerosal. It comes in a prefilled syringe. Only the inactivated form of any flu vaccine should be given for DBA patients as well as family members. The nasal mist (given up the nose) is live virus and should not be used. The vaccine has been given to neutropenic cancer patients receiving chemotherapy.

The goal is prevention so vaccination is preferred to the actual virus. If the DBA patient is not a candidate for the H1N1 vaccine then the members of the household should be vaccinated in hopes of protecting the patient.

If your child should get the H1N1 virus, Tamiflu (Oseltamivir) and Relenza (Zanamivir) are the only antivirals to help reduce the time that you have the virus and minimize the symptoms. It does not "cure" the virus. Relenza is not to be used if you have asthma or lung disease and are younger than 7 years of age.

In 1976, an earlier type of swine flu vaccine was associated with cases of Guillian-Barre Syndrome (GBS). Since then, flu vaccines have not been clearly linked to GBS. For more information please on GBS, see www.cdc.gov/h1n1flu/vaccination/gbs_qa.htm.

For more information on the H1N1 flu and the vaccine go to:
www.flu.gov
www.cdc.gov/h1n1flu/
For high risk students:
www.flu.gov/professional/school/k12techreport.html

Sincerely,
Ellen Muir, DBA Nurse

Submitted with direction from Dr. A. Vlachos, M.D.



Ellen Muir, DBA Nurse

Gift of Appreciated Stock

Turning stock market gains into the search for a cure for DBA

Everybody wins when you make a gift of appreciated stock to the Diamond Blackfan Anemia Foundation (DBAF). Your gift of stock is reinvested in efforts to find a cure, and it qualifies for an immediate tax deduction based on the full fair-market value.

How it works

When you make a gift of appreciated stock to the DBAF:

- Your gift can be placed into our charitable funds account. For example, you can use your gift to create a Donor Advised Fund in your name, in the name of your family or business, or in honor of any person or organization that you choose. This type of fund allows the donor to stay involved in the determination of how the gifted fund is put to use.
- Your gift can be placed into an endowment that is invested over time. Earnings from your fund will then be used to meet the needs of the DBAF. Your gift— and all future earnings from your gift— become a permanent source of DBAF capital, allowing our loved ones to continue to benefit.

- Your gift qualifies for a tax deduction based on the full market value of your stock, therefore allowing you to avoid the capital gains tax that would otherwise arise from the sale of this stock.

More benefits

Giving appreciated stock through the DBAF is popular among a range of givers including individual investors, families and businesses.

By giving stock through the DBAF, capital gains taxes can be avoided that would otherwise be due as a result of its sale. Additionally, your gift allows for the establishment of a charitable fund that ultimately benefits the people you care about the most.

For more information contact Cara Breidster at cbreidster@dbafoundation.org. Thank you for helping the DBAF work to meet the needs of our families through your gift of appreciated stock!

A Word From the Board

by Jacy Downey, Editor

Greetings fellow DBA families! I hope your holidays were pleasant and you and your families found everything you wished for under the tree. I know ours were especially blessed, as we were overjoyed to be at home. Not long ago we spent both Thanksgiving and Christmas dislocated from our home and loved ones as our middle child underwent a bone marrow transplant on November 25th, 2008 in Minneapolis, Minnesota. We are happy to report that his big brother's marrow has found a nice home and he is producing platelets, white blood cells and the beloved red blood cells. Out of all of the Christmas mornings in recent years, none will soon compare to this past one as the hope we feel for our son's future has never seemed brighter.

Bone marrow transplants are risky yet rewarding endeavors. Not an experience that every person with DBA could or should take on. We all have our own imperfect treatments that we struggle with. The love/hate relationship of steroids or blood transfusions and chelation is real and constant, but we must admit that currently this is all that we have to keep our loved ones alive.

But what if someday there were better options? What if a child born with Diamond Blackfan Anemia had the promise of a treatment that was more tailored to treat this rare disorder? Treatment that had minimal side effects and less invasive deliveries. What if...

Today there are at least five studies looking for funding that could possibly bring our dreams for our loved ones a little bit closer. Right now, knocking at our door, are at least five teams of researchers with big brains and even bigger dreams, yearning to uncover just a little more about our little known disease. They need us to help them. They need us to fund their research so that the hope of a better life for our parent, our wife, our husband, and our children can become a reality.

Personally, I cannot stomach the thought of turning these scientists away. I know my son's battle with DBA is far from over, and though his story may read differently than yours, I will never stop doing what I can to ensure that our little known disease becomes more understood. What about you?

A handful of families come through year after year. They move mountains to provide funding for the DBA Foundation. Some of our

DBA families have held hugely-successful golf outings where they have repeatedly asked their communities to come forth and give on their child's behalf. Other families have bike rides and bake sales or send out compelling letters, campaigning for dollars for DBA. It may not be much, but at my house, among other small events, we ask for donations to the DBAF from our friends and families instead of Christmas and birthday gifts. Dance-a-thons, car washes, 24-hour bowling events... How could YOU help fund these studies? Have you taken a minute to evaluate what kind of fundraiser you might feel comfortable with?

In closing, we, at the DBAF, know that times are incredibly tough right now. Layoffs and losses in the market. High grocery costs and heating bills. If this just isn't the right time to reach into your pockets or plan a fundraiser, then please know that the fellow members of your DBA family wish only the best for you and yours and understand that your contribution will have to wait. But if the time is right, please feel free to reach out for help, ideas or inspiration in planning the event that is comfortable for you. One simple way to do your part is to jump on board with Joe and Casey in their heroic efforts to raise money for DBA. A simple email blast or letter-writing campaign to friends and family about Joe's Ironman and Casey's Relay will help them reach their goals and benefit us all. Additionally, please take the time to look into your employer's company matching program. In some instances your efforts can be doubled by simply completing a form. We also encourage you to ensure that the DBAF is listed among the candidates on your local United Way list of beneficiaries. Please contact Dawn at dbaumgardner@dbafoundation.org or dbafoundation@juno.com for aid in this process. A visit to our website provides information and links to many avenues to join the fundraising effort including the DBA awareness wristbands, Mary Kay for DBA and the www.dbaftavel.com website.

As I come upon my one year anniversary as a member of the DBAF Board, I reflect upon all that I have experienced both as a mother of a child with DBA and also as a representative of the greater DBA family. I know I speak for the other members of the Board when I say that it has been an honor to work on your behalf. Please feel confident that we are determined to be good stewards of your donated dollars.

Board Members

Dawn and Peter Baumgardner, West Seneca, NY

Kathi Vroman, Atlanta, Georgia

Rebecca Bromet, Chicago, Illinois

Anita Shier Bruton, Topanga, CA

Cara Breidster, Indianapolis, Indiana

Carol Mancuso, Cleveland, Ohio

Jacy Downey, Indianola, Iowa

Research Director

Steven R. Ellis, Ph.D

DBAF Research Director

Professor

Department of Biochemistry and Molecular Biology

University of Louisville

Louisville, KY

Friends of DBAF Golf Outing and Silent Auction

By Jim and Carol Mancuso

One article of each newsletter will be devoted to including a detailed description of a past fundraiser in an effort to provide instruction and inspiration to anyone who might be searching for the perfect fundraising "fit". Please take a moment to read the step-by-step account of how the Mancuso Family organizes their annual benefit.

Once again, on September 12, 2009, we held another successful golf outing and silent auction. By raising money we are proud to contribute to the search for a cure for Trevor, our son, and others with DBA.

Hosting this annual event has become a gratifying as well as humbling experience for our family. Each year we are amazed by the financial, physical and emotional support we receive from those around us. In the end, the time and energy of all involved are well rewarded.

There is no right or wrong way to proceed with an event, but we have found through trial and error what works for us. Nothing really happens until we do something, so that is where we begin...

Our event takes place in September, so approximately 1500 letters are sent out in late January or early February asking for donations for the silent auction. In addition to mailings, we ask local businesses and friends to consider who else they may know and what they might be able to offer. We also always have a donation letter with us...you never know who you might run into!

At about 6 months in advance, we send out "save the date" cards. The formal invitations for our golf outing and silent auction are usually sent out about 2 - 3 months before the event. We mail them to anyone and everyone with a note to extend the invitation in hopes of maximizing attendance as space allows. Included in the invitation is information about why we are doing the event, details on how to donate or become a sponsor, fees, deadlines and contact information. We offer a reduced price for those who sign-up a month prior to the deadline. The invitation also provides details about the DBA Foundation as well as the DBAF website address.

As donations for the auction come in they are logged and placed into themed baskets. Additional items are then purchased as filler to complete the themed baskets. Often, there are people who donate cash to be used to help cover miscellaneous costs. Some of these funds are used to complete the baskets. All baskets are then inventoried, designed and then wrapped with cellophane and bows. Ample time is spent arranging the baskets as we have found that the better looking the basket, the higher the bid. (Please note: most of the baskets are not complete until about a week before the event.) We then create description sheets to provide more detailed information about the items. The night of the event, this information is displayed along with the basket and bid sheet. Our autographed items and those of higher value have a higher starting bid as well as larger increments.

Each year we have at least one piece of artwork done by Trevor to auction off- a great way to show off his talent and bring the auction back to a personal level!

We keep our attendees coming back each year by focusing primarily on cost, activities and atmosphere. The admittance fee gets the golfer a boxed lunch, 18 holes with GPS cart and free beverages on the course along with dinner, desert and beer/soda/coffee during the auction.

We advertise by word of mouth, local newspapers that do free "around town" advertising as well as local radio stations that offer free airtime to spotlight community events. Additionally, most of our guests have been with us since we started this event, bringing new people with

them each year. We have found if we keep them happy they will bring others with them the following year!

Hole sponsors get their names on a banner at registration as well as on the GPS carts leading up to each hole. Our banner also displays the names of those sponsors who give \$250 or more. During dinner and throughout the evening, the banner is mounted in the banquet room to allow our generous guests to continue to be honored.

Registration begins about one hour before the start of the event and includes a sign-in sheet, pick-up station for boxed lunches and features a putting contest. Golfers that have not already joined a team are put into foursomes as they register. We like doing shotgun starts so that everyone gets off the course and into the auction at about the same time. We offer cocktails and appetizers until dinner is served to keep our guests happy as they get an early view of our silent auction items.

During registration and throughout the entire event, Trevor is very visible and welcomes everyone. Jim even drives Trevor around the course so that he can drive and/or put with every team. Our guests just love it!

During dinner, Trevor offers a few words and thanks everyone for coming. Jim usually gives a little speech of thanks, talks about current research, gives updates on Trevor's condition and of recent activities of the DBAF Board. At this time we also hand out prizes for our golf winners. We have trophies that are given to us at "cost" for each foursome group that wins the overall event, has the longest drive and is closest to the pin.

Throughout the evening we sell raffle tickets for one specific item. This past year it was a "Barrel of Booze" which consisted of various donated beverages, ensuring the raffle would be a purely profitable highlight of the evening. Last year we raffled off flower pots painted by Trevor, which had been showcased throughout the event as table decorations. In working with a local green house to purchase the flowers at "cost", again we were able to keep our operating expenses down. The painted flowerpots brought Trevor back into the mix, making the event more personal and focused our guests on the reason that they had gathered. Each place setting is given a favor as an additional "thank you" for being there, and this year we chose to give away note cards featuring Trevor's artwork. This was a big hit, once again reminding everyone of the purpose of the occasion.

Thank yous are a "must". We list our contributing sponsors, include pictures from the festivities and announce the grand total that was raised. We also include the date for next year's event and contact information. We are pleased to announce that on September 18, 2010, we will be holding the 4th annual **Friends of DBA Silent Auction and Golf Outing** at Cherokee Hills Golf Course in Valley City, Ohio.

As Trevor's parents, we must include a mention of how holding an annual fundraiser has affected our family on a personal level. We are humbled by the kindnesses of those around us who have come to look forward to our event each year. With all the ups and downs, issues and concerns we continue to experience much goodness and many happy moments in the process of organizing this event. Each year is an adventure, and we look forward to a day when we are holding fundraisers not to fund research looking for a cure but to help each and every DBA family get access to that cure!



The Refrigerator Door

Do you have a beautiful picture hanging in your home that you would like to share? The DBA Newsletter is requesting submissions of your or your children's artwork, photography, poems, short stories, etc. to include in this publication. Please send a copy of your work to Jacy Downey at 1200 W. Euclid Indianola, IA 50125 or electronically to jdowney@dbafoundation.org.

The poem below was written by Matilda O'Sullivan of Australia. Although she did not win the contest, we believe she certainly will win the hearts of our readers. It was submitted by Jessica Bond. Matilda is the ten year-old cousin of Jessica's son, Angus Bond, who is 18 months old and has DBA.

Diamond Black Fan Disease

I have a small little cousin who can handle a dozen!
His name is Angus Bond with a blood disease above and beyond!
It is extremely sad but I am glad he will live and learn to give!
When times get really hard and rough Angus always remains strong and tough!
When he is good he gets a spoonful of honey but if I win I will choose the money!
I would like to use the prize money to donate and find a cure and then celebrate!
This disease is very rare, it needs lots of money to help repair!

By Matilda O'Sullivan 4B

Fundraisers

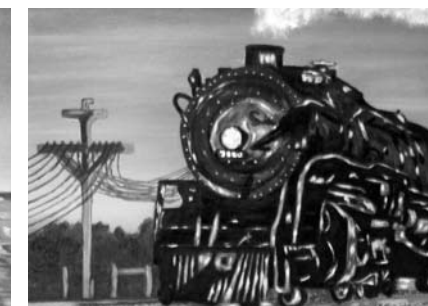


The Lenore Fundraiser



The Downey Fundraiser

Artwork by Trevor Mancuso



Upcoming DBA Fundraisers

Galactic Bowling

April 17, 2010
7:00 pm - 10:00 pm
Starlite Lanes
Grand Haven, Michigan
Hosted by the Lenore Family

Re-Prom 2010

May 1, 2010
7:00pm-12:00am
Warren County Fairgrounds
Indianola, Iowa
Hosted by the Downey Family

The Cape 13 Relay

May 1 and 2, 2010
Quincy, MA to the tip of Cape Cod
Hosted by Casey Leonard

Janus Charity Challenge

2010 Ironman Coeur d'Alene
June 27, 2010
Coeur d'Alene, Idaho
Hosted by Joe Crelier

Friends of DBAF

4th Annual Golf Outing and Silent Auction
September 18, 2010
Cherokee Hills Golf Club
Valley City, Ohio
Hosted by the Mancuso Family

DBAF Fundraisers of the Past

Family Dance for DBA
Carwash
Dance for DBA
Bike Ride
5K Run/Walk
Birthday Parties (donation in lieu of a gift)
Mini Golf
Golf Outing
Restaurant "Tip Night" for DBA
Silent or Live Auction
Bowl-a-thon
Costume Party
Letter-Writing Campaign
Trialthalon
Extreme Races
Bake Sale
Craft Sale
Hat/Jeans Day at Work or School
Mary Kay Special for DBA
Dbaftravel.com Website
DBA Awareness Wristbands

Meet The Scientific Advisory Board



Steve Ellis,
DBAF Board of Directors

In each issue of the DBA Newsletter we will spotlight a member of the DBAF Scientific Advisory Board. The Foundation is very lucky to have an esteemed group of medical professionals who are dedicated to working with the Board to achieve our mission. The inaugural member to provide an autobiography is Research Director, Steve Ellis.

Hi everyone, my name is Steve Ellis and I was born and raised in Lauderdale, a small suburb of St. Paul, Minnesota. I received my B.S. in Biochemistry from the University of Minnesota in 1977 and a Ph.D. in Biochemistry at the University of Iowa in 1983. After a postdoctoral fellowship at Southwestern Medical School in Dallas from 1983-1987, I joined the faculty of the Department of Biochemistry at the University of Louisville School of Medicine where I have been ever since.

My interest in ribosomal proteins began as a graduate student at the University of Iowa. My first publication as a graduate student came in 1979 describing our studies on ribosomal protein S1. I remember being invited back to the University of Iowa in the late 80's upon the retirement of my mentor, Tom Conway. At that point I gave a talk on the properties of ribosomal protein S2. So in 10 years I had moved from ribosomal protein S1 to S2...not exactly the fast track to fame and fortune in the scientific world! Studies on ribosomal proteins really had their heyday in the 1960s: with the remarkable studies of Masayasu Nomura and colleagues who were able to take small ribosomal subunits apart (21 proteins and one RNA), purify each component, and then put them back together again to give a functional subunit. While not recognized with the recent Nobel Prize in Chemistry for studies on the three dimensional structure of the ribosome, Nomura's studies most certainly pioneered the way for this remarkable achievement.

Ah, but back to my story. By the 70's, 80's, and 90's ribosomal proteins had lost their luster and I, for one, was barely able to keep my research on ribosomal proteins afloat. By the new millennium, conditions were getting desperate. I was given a life line by the Kentucky Lung Cancer Research Program which funded a proposal I had submitted suggesting a link between the loss of function of ribosomal protein S0 (yes, our studies by this time were regressing along the ribosomal

protein numbering scheme) and lung cancer. At the time, the idea that a ribosomal protein might be involved in a human disease hadn't really taken root so the KLCRP was taking a significant risk supporting these studies. While we were unable to demonstrate that RPS0 functions as a tumor suppressor in lung cancer, these studies did pave the way for our work on Diamond Blackfan Anemia which I think will ultimately come full circle and bring us back to the roles for ribosomal proteins as tumor suppressors.

Our work took a quantum jump forward in the early 2000s. Not only had we vaulted ahead from ribosomal protein S0 to S19, but now our research on ribosomal proteins caught the eye of a small but passionate group of physicians, scientists and parents who had a vested interest in obtaining knowledge on the functions of ribosomal proteins and the consequences of the loss of these functions on human growth and development. Thus, my entry into the DBA world!

In looking back at this fateful time, I cannot believe my good fortune. I was among a group of investigators supported by the National Heart Lung and Blood Institute for research into the molecular underpinning of DBA and other bone marrow failure syndromes. Furthermore, I have been able to meet yearly with a remarkable group of clinicians, scientists, and lay individuals interested in, or studying DBA at the International Consensus Conferences in New York City sponsored by the Daniella Maria Arturi Foundation with support from the Diamond Blackfan Anemia Foundation. It was my desire to be a bigger part of this "DBA family" that caused me to reach out to the Diamond Blackfan Anemia Foundation and apply for the position of research director. While an unknown from Kentucky working with yeast was not exactly what the DBAF Board had in mind for a research director, they nevertheless took a chance and gave me the position.

As research director, I have had the opportunity to visit Camp Sunshine and actually meet children afflicted with DBA. While their smiles and excited demeanors at Camp tend to mask some of the debilitating aspects of this disease, listening to tearful parents recount the hardships of living with DBA is heartbreaking. It also re-enforces a point made to me on several occasions by Dawn Baumgardner and Kathi Vroman, two members of the DBAF Board, that despite my interest in the underlying molecular basis of DBA, we are in this for those affected by DBA on a daily basis and that improving the quality of life for individuals affected by DBA should be our focus. I want to close by saying that I take their words to heart and find them a major motivating influence in my life.

The Tooth Fairy "paid" a visit to **Amelia Lamb**, who lost her first two teeth!

Cameron Lanore reports having one wish come true, one record broken, and one goal to attain. The Michigan State University Spartan Football Team and Make-A-Wish teamed up to give Cameron and his younger brother, Bryce and their parents, Justin and Tammi a very special MSU weekend. Cameron also enjoys bowling and recently bowled his highest game of 130 during league play (without bumpers!). His goal...beat his mom!!

Jenn and Shawn Morrison welcomed their daughter, Hannah (6 pounds 3 ounces) into the world on Nov. 18, 2009 at 6:24 am. Congratulations Jenn and Shawn and big sister, **Andie**.

KUDOS *Continued from Page 8*

Makayla Pereira, big sister of **Jack Pereira**, was a recipient of the *Ontario Junior Citizen of the Year Award*. The awards recognize outstanding youth who are making a difference in their communities. Makayla has worked tirelessly to organize and raise funds for worthy causes through Walks and bracelet sales. "Makayla's DBA Bracelets" have raised \$3000 and counting for DBA!

John Shannon IV is featured in the Delmarva Blood Bank's corporate recruiting DVD. He also danced in his first Holiday Dance Recital and clearly loves the stage!

Jillie Snyder and her big sister, Jaycee, love cheerleading. Jillie is an All-Star cheerleader for Mid West Cheer Elite. This is Jillie's first year competing, and Jaycee's third year. Jillie does an awesome job and has perfected her cart wheels and round offs!

2009 Research Review

Steve Ellis, DBAF Research Director

In early October of this year I received a number of e-mails regarding the announcement that the Nobel Prize in Chemistry was awarded to a trio of researchers (Professors Venkatraman, Steitz, and Yonath) whose work had led to the determination of the three dimensional structure of the ribosome. Many of the e-mails wondered what implications this recognition had for DBA research. My response was generally "not a whole lot", which I'm afraid likely surprised some of my questioners. But, for the most part, I stand by this initial response. It is not that I do not believe solving the three dimensional structure of the ribosome was a remarkable achievement, it is just that much of the work on which the Nobel Prize was awarded, was performed and published in the late 1990's and early to mid-2000's, so the impact of this research had already been incorporated into our ribo-centric view of Diamond Blackfan Anemia.

The structure of the ribosome is particularly important when considering newly identified DBA genes. Over the past year we have witnessed the discovery of three additional DBA genes, Rpl5, Rpl11, and Rps7. These gene discovery efforts were spearheaded by Dr. Hanna Gazda (Children's Hospital Boston) in collaboration with a number of investigators throughout the world. Two of these proteins, Rpl5 and Rpl11, form a subcomplex on the ribosome with one of the ribosomal RNAs (5S rRNA). The Rpl5, Rpl11, 5S rRNA subcomplex is the first functional unit of the ribosome implicated in DBA and it is worthwhile noting that patients with mutations in RPL5 tend to have more severe forms of the disease with an increased number of congenital malformations including cleft lip and/or palate. Mutations in RPL11, on the other hand, are associated with an increased incidence of thumb malformations. These observations are extremely important because they are the first example of what are called genotype/phenotype relationships in DBA. The importance of establishing genotype/phenotype relationships is that it may ultimately be possible to use genetic information to predict a patient's probability of responding to treatment modalities (for example steroids) or hopefully, even the probability that a patient may go into spontaneous remission. The support for these gene discovery efforts was provided, in part, by the DBA Foundation.

The Rpl5, Rpl11, 5S rRNA subcomplex may also fit into another aspect of DBA pathophysiology since Rpl11, if not all 3 components of this

Atleigh Whitman graduated from John Overton High School, and she is currently studying to be a radiologist tech at Columbia State Community College.

Marcus "Ty" Wilkins possesses a servant's heart. Even with all of his recent struggles, Ty worked alongside other home school children making sandwiches and filling bags for St. Vincent De Paul.

Jennifer and **Jamaal Williams** welcomed their son, **Michael Ian** (7 pounds 1 ounce) into the world on Dec. 12, 2009 at 2:24 am. Congratulations **Jen** and **Jamaal** and big brother, **Calvin**.

subcomplex, appears to play a critical role in signaling abortive ribosome assembly to cell fate decisions which are increasingly thought to play a role in clinical features of DBA. The role of Rpl11 in this signaling pathway which leads to activation of p53, a key regulator of cell fate decisions, was published in April from the laboratory of George Thomas at the University of Cincinnati 2. These studies came on the heels of reports from a growing number of animal models for DBA which demonstrated that p53 activation was necessary for the developmental defects observed in these animal models 3-5. Although not proven, this pathway linking abortive ribosome assembly to p53 activation may explain the death of erythroid progenitors in the marrow of DBA patients. Understanding the pathway by which abortive ribosome assembly leads to enhanced cell death in erythroid progenitors remains an area of intense interest in the DBA research community, since this pathway could represent a therapeutic target. Again, I feel it is important to point out that the first of these animal models to establish the connection between RPS19 mutations and p53 activation was supported, in part, by funds from the DBA Foundation 4.

As our understanding of the underlying molecular and genetic basis of DBA continues to grow, so does our appreciation of nontraditional forms of DBA. Two reports over the past year have brought attention to the concept of nontraditional DBA 6,7. The first is the DBA clinical consensus document published in 2008, which details diagnostic criteria for DBA and the challenges posed by nontraditional forms of DBA 7. (A link on the DBAF website provides navigation to this document.) In this latter regard, it is known that certain family members of DBA patients have the same mutation as the affected individual without manifesting the bone marrow failure, but perhaps having some of the minor constellation of congenital anomalies associated with DBA. Thus, DBA as a "syndrome" may be more widespread than originally thought.

Anyone wishing to learn more about developments in DBA research over the past 12-18 months is encouraged to turn to a recent review in *Current Opinion in Pediatrics* by Lipton and Ellis 8. As Director of Research, it has been my great pleasure to be a part of the search for a cure for your loved ones. Please know how important and valuable your fundraising dollars are to completing our common mission.

KUDOS

Let's celebrate together! Please email Jacy at jdowney@dbafoundation.org with marriages, births, graduations, promotions, milestones, and any other event that your family would like to share.

Kyle Baumgardner will be graduating with honors from Bishop Timon - St. Jude High School in May 2010. He recently was awarded a Dean's Scholarship to Canisius College, where he will be studying Computer Science.

Sinclare Broadhurst turned the big "7" on Feb 12, 2010!

Bowen Downey celebrated his first transplanniversary on Nov 25, 2009!

Dean Edwards has been studying karate for the past three years. He worked extra hard to move up with his class after being sick with the H1N1 virus. Dean earned his blue with white strip belt along with 3 patches.

2009 Research Review *Continued from Page 9*

1. Gazda HT, Sheen MR, Vlachos A, et al. Ribosomal protein L5 and L11 mutations are associated with cleft palate and abnormal thumbs in Diamond-Blackfan anemia patients. *Am J Hum Genet.* 2008;83:769-780.
2. Fumagalli S, Di Cara A, Neb-Gulati A, et al. Absence of nucleolar disruption after impairment of 40S ribosome biogenesis reveals an rpL11-translation-dependent mechanism of p53 induction. *Nat Cell Biol.* 2009;11:501-508.
3. McGowan KA, Li JZ, Park CY, et al. Ribosomal mutations cause p53-mediated dark skin and pleiotropic effects. *Nat Genet.* 2008;40:963-970.
4. Danilova N, Sakamoto KM, Lin S. Ribosomal protein S19 deficiency in zebrafish leads to developmental abnormalities and defective erythropoiesis through activation of p53 protein family. *Blood.* 2008.
5. Uechi T, Nakajima Y, Chakraborty A, Torihara H, Higa S, Kenmochi N. Deficiency of ribosomal protein S19 during early embryogenesis leads to reduction of erythrocytes in a zebrafish model of Diamond-Blackfan anemia. *Hum Mol Genet.* 2008;17:3204-3211.
6. Anur P, Nemecek ER, Kurre P. The evolving spectrum of ‘non-classical’ Diamond-Blackfan anaemia—a case of eADA positive pancytopenia in a young adult. *Br J Haematol.* 2009;145:428-430.
7. Vlachos A, Ball S, Dahl N, et al. Diagnosing and treating Diamond Blackfan anaemia: results of an international clinical consensus conference. *Br J Haematol.* 2008;142:859-876.
8. Lipton JM, Ellis SR. Diamond Blackfan anemia 2008-2009: broadening the scope of ribosome biogenesis disorders. *Curr Opin Pediatr.* 2009.

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Pilot Study of Lenalidomide

Dear DBA Patients and Families

We are pleased to announce the opening of the “Pilot Study of Lenalidomide (Revlimid) in Adult Patients with Red Blood Cell Transfusion-Dependent Anemia” at the Stanford University Medical Center.

The rationale for evaluating lenalidomide in DBA comes from the study of an acquired form of bone marrow failure named myelodysplastic syndrome (MDS). Although DBA is inherited, and MDS usually develops in older adults, they share several clinical and laboratory features: poor production of red blood cells often leading to a need for frequent blood transfusions, and a somewhat increased risk of developing acute leukemia. Iron overload from chronic red blood cell transfusions is a common and serious problem.

Mutations in more than 10 ribosomal proteins have now been identified in greater than 50% of DBA patients and are now implicated as the cause of the disease. In a very important report published in *Nature* in 2008, Dr. Benjamin Ebert and colleagues at Harvard discovered that loss of the gene encoding ribosomal protein S14 was critical to the cause of a form of MDS called 5q- syndrome. In this subtype of MDS, a region of the long arm of chromosome 5 is missing, and ribosomal protein gene S14 is located in this missing segment. This was an unexpected and important finding, since it was the first time that a common biologic link was found between DBA and MDS.

In patients with 5q- MDS who depend on red blood cell transfusions, the oral medication lenalidomide (Revlimid) can eliminate the need for transfusions in 70% of subjects. Given the outstanding red blood cell response rate to lenalidomide in 5q- MDS in which loss of ribosomal protein S14 has emerged as an important discovery, there is a strong reason for investigating lenalidomide in DBA in which ribosomal protein deficiency is also linked to impaired red blood cell production.

The following are some of the basic inclusion and exclusion criteria for this pilot trial:

Inclusion Criteria

1. Age \geq 18 years at the time of signing the informed consent form.
2. Red blood cell transfusion-dependent with a requirement of at least one unit of RBCs per month for the 2 months prior to study enrollment (e.g. 2 units/8 weeks)
3. If applicable, ongoing therapy with a stable or decreasing dose of prednisone $<$ 60 mg/d or corticosteroid equivalent, for which there has been no treatment-related improvement in RBC transfusion requirements for at least 2 months prior to study entry
4. Laboratory test results within these ranges:
 - Absolute neutrophil count \geq 1500/mm³
 - Platelet count \geq 100,000/mm³
 - Serum creatinine \leq 2.0 mg/dL
 - Direct bilirubin \leq 1.5 mg/dL
 - AST (SGOT) and ALT (SGPT) \leq 2.5 x upper limit of normal
 - Disease free of prior malignancies for \geq 5 years with exception of currently treated basal cell, squamous cell carcinoma of the skin, or carcinoma “in-situ” of the cervix or breast.

5. Females of childbearing potential must have a negative serum or urine pregnancy test.

6. Able to take aspirin (81 - 325 mg) daily as prophylactic anticoagulation (patients intolerant to ASA may use warfarin or low molecular weight heparin).

Exclusion criteria

1. Pregnant or breast feeding females. (Lactating females must agree not to breast feed while taking lenalidomide).
2. Use of any other experimental drug or therapy (excluding steroids) specifically used for DBA within 28 days of baseline including metoclopramide, leucine, danazol, or other hormonal therapy.
3. Clinically significant anemia due to factors such as iron, B12, folate deficiencies, autoimmune or hereditary hemolysis, or gastrointestinal bleeding.
4. Known hypersensitivity to thalidomide.
5. Concurrent use of other anti-cancer agents or treatments.
6. Known positive for HIV or infectious hepatitis, type A, B or C.

Please refer to the website link: <http://www.clinicaltrials.gov> and enter the search term “Diamond-Blackfan Anemia and lenalidomide” for full eligibility criteria and additional trial information.

NOTE: BECAUSE THIS STUDY IS CURRENTLY ONLY BEING CONDUCTED AT STANFORD, WE HAVE FUNDS TO COVER PATIENT TRAVEL EXPENSES.

CONTACT INFORMATION

Principal Investigator: Dr. Jason Gotlib, Stanford University School of Medicine

Co-Investigator: Dr. Bertil Glader, Stanford University School of Medicine

Scientific Co-Investigators:

Dr. Hanna Gazda, Boston Children’s Hospital/Harvard
Dr. Benjamin Ebert, Brigham and Women’s Hospital/Harvard

We appreciate the support and commitment of Drs. Adrianna Vlachos and Jeffrey Lipton and their leadership of the DBA Registry in making this study possible.

Please contact our study nurse, Andrea Linder:

TEL: 650-725-4047

FAX: 650-723-1269

Email: alinder@stanford.edu

Also, feel free to contact Dr. Gotlib by email: jason.gotlib@stanford.edu

Pilot Study of the Amino Acid Leucine

Greetings DBA Families,

We are excited to be able to provide the details of the leucine trial as a new possible treatment option for individuals with Diamond Blackfan Anemia!

Title: A Pilot Study of the Amino Acid Leucine in the Treatment of Patients with Transfusion- Dependent Diamond Blackfan Anemia

Principal Investigator:

Adrianna Vlachos, MD (Schneider Children's Hospital, New Hyde Park, NY)

Co- Investigators:

George Buchanan, MD/ Zora Rogers, MD (University of Texas Southwestern Medical Center, Dallas, TX)

Steven R. Ellis, PhD (University of Louisville Dept. of Biochemistry and Molecular Biology, Louisville, KY)

Bertil Glader, MD, PhD (Stanford University, Palo Alto, CA)

Jeffrey M. Lipton, MD, PhD (Schneider Children's Hospital, New Hyde Park, N)

Colin Sieff, MD (Dana Farber Cancer Institute, Boston, MA)

Mitchell Weiss, MD, PhD/ Kim Smith-Whitley, MD (The Children's Hospital of Philadelphia, Philadelphia, PA)

Inspiration for this pilot study derived from an instance when leucine was given to a patient and resulted in a positive outcome that was subsequently reported in the literature. To date, more than 5 patients have been followed using the same protocol. Partial response was found in 4 of the 5 patients. The concept for the use of the amino acid leucine as a therapeutic agent for DBA is based upon the idea that supplementation may help to "turn on" the protein machinery of the cells. As DBA is caused by a defect in one of the ribosomal proteins in some patients, leucine supplementation may result in increased ribosomal protein production.

This study will include 50 transfused DBA individuals, measuring amino acid levels at different time points during the study. Since this is a multicenter trial, there will be no travel involved. Your hematologist,

however, needs to agree to drawing and shipping the blood for the leucine levels (paid for by the study). The other lab tests are considered "standard of care" and will continue to be paid for by your insurance company. The objectives will be:

Primary:

1. To determine the feasibility (possibility and reality) of administering the amino acid leucine to patients with Diamond Blackfan Anemia (DBA) who are red cell transfusion-dependent.
2. To determine the efficacy of leucine to produce an erythroid response (to see if hemoglobin goes up) in patients with transfusion-dependent DBA.

Secondary:

1. To determine the safety profile of leucine in DBA patients.
2. To determine the pharmacokinetics (blood levels) of leucine administration in DBA patients.
3. To determine if patients with DBA are deficient in branched chain amino acids (such as leucine).

Eligibility:

Inclusion Criteria (those who can participate) -

1. Signed informed consent.
2. Age greater than 2 years.
3. Documented diagnosis of Diamond-Blackfan Anemia.
4. Documented transfusion-dependence defined as requiring regular RBC transfusions every 3 to 5 weeks for reason of persistent anemia for at least the last 6 months.
5. Total bilirubin ≤ 1.5 x upper normal limit (UNL) and alanine aminotransferase (ALT) ≤ 2.5 x UNL, creatinine ≤ 1.5 x UNL.
6. Negative serum pregnancy test (if patient is a menstruating female) and has documentation of adequate contraception.
7. Registration in the Diamond Blackfan Anemia Registry (DBAR) will be offered but not required for study enrollment.

Exclusion Criteria (those who cannot participate) –

The CDC partners with Four National Resource Centers for the Treatment of DBA:

Schneider Children's Hospital

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Albert Einstein College of Medicine
Head, Bone Marrow Failure Program
Director, Diamond Blackfan Anemia Registry

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1-877-DBA Nurse

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Southwestern Medical Center
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Phone: 1-617-355-8246

Pilot Study of the Amino Acid Leucine *Continued from Page 12*

1. Allergy to amino acids.
2. Diagnosis of an inborn error of amino acid metabolism (such as maple syrup urine disease).
3. Known sulfite sensitivity. Sulfites are additives to some foods and medications which are used as preservatives. Sensitivity may include facial flushing, difficulty breathing, hives and/ or swelling. Some of the foods that sulfites are found in include: dried fruits (excluding dark raisins and prunes), bottled lemon juice (non-frozen), bottled lime juice (non-frozen), wine, molasses, sauerkraut (and its juice), grape juices (white, white sparkling, pink sparkling, red sparkling) and pickled cocktail onions
4. Evidence of liver failure.
5. Pregnancy or lactation or unwillingness to take contraceptives.

The leucine will be supplied in a powder form with a scoop for measuring the prescribed amount. The leucine will then be diluted in a liquid – water, juice, milk, for example. The patient will take one-third of the diluted powder three times per day for a 9 month period. Blood will be drawn to evaluate leucine levels at certain time periods during the study and can be coordinated with blood transfusions. Information will be ascertained to monitor for side effects as well as blood collected for CBC, differential, reticulocyte count and blood chemistries, which evaluate liver and kidney function, and ferritin levels. These are tests that are usually being monitored already, especially if the patients are taking Exjade or Desferal.

This is a very exciting time for DBA as there are new findings and

Thank You to Our Families and Friends

The Diamond Blackfan Anemia Foundation, Inc. is proud of our accomplishments in 2009! To continue funding 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 who have supported the DBAF in 2009. Together, our wonderful families have provided the DBAF the opportunity to accomplish our mission.

We strongly encourage ALL our families to get involved. We need your partnership 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
Lindsey Baur
Heather Beilman
Elizabeth Bell
Nathaniel Boatman
James Bohuski
Alexandra Braue
Sinclare Broadhurst
Caitlyn Rose Brown
Jake Brunette
Gabriel Bruton
Jacob Buckmaster

Chasity Chandler
Shannon Childs
Pascal Cheung
Gail Coughlin
Meaghan Covington
Peggy Craft
Bowen Downey
Dean Edwards
Kimberly Edson
William Fair
Tori Flavahan
Derek Free
James Thomas Garrett
Kevin Gately
Shayna Goldrich
Peyton Green

Erik Harrison
Angela Joyce
Michael Joyce
Carole Kauffman
Jennifer Kleiber
Bria Koch
Amelia Lamb
Cameron Lanore
Bailey Lightner
Jaclyn Mancuso
Trevor Mancuso
Samuel Mancuso
Sam Marchese
Sam Marchese
Shelly Marquer
Paige Mauch
Nancy McSweeney

Jeannie Michael
Kylie Monica
Dylan Nape
Audrey Nethery
Jack Pereira
Kelly Pierce
William Plourde
Brianna Ramsell
Emily Reimer
John Shannon IV
Coleson Shaw
Zachary Shaw
Sarah Williams Shelby
Jillie Snyder
Arien Stoeckle
Liana Svensson

Ryan Spring
Katie Trebing
Christopher and Matthew Vroman
Andreas Wagner
Dan Wagner
Sean Ware
Atleigh Whitman
Marcus (Ty) Wilkins
Lola Willford

In Loving Memory of:

Lakshmi Desai
Deana Valentino
Brett Wiegref
Keir Zangrando

Dream...Believe...Achieve

"Simply, the thing I am shall make me live," - William Shakespeare.

Words are just words until people make them mean something, and people are just people until we come together. Many of you reading this article have Diamond Blackfan Anemia. All of you have had to deal with the stress it brings. Let's face it, no one likes the needles, going to the doctors, or knowing they have a blood disorder.

At times, it is hard to stay positive, especially when some genius in the hallway just made the scientific discovery that you're short or have puffy cheeks. I used to let these comments bother me. Then I realized that I wouldn't be me if I didn't have DBA. DBA is what makes me unique. Honestly, without it I'm just the same as everyone else. Now, I'll crack a short joke myself. When the person who pointed out my height doesn't have anything clever enough to say back, I laugh at how dumb they just made themselves look. Diamond Blackfan Anemia is not something that needs to be overcome but rather conquered.

Just because we have DBA doesn't mean we're limited. DBA is not a barrier between us and everyone else. Just because you have a blood disorder, doesn't mean you won't succeed. It doesn't even mean you'll have to work harder. It just means we know the reality of life. We know things aren't always perfect, and there's not much we can do about it. We just have to keep on living our lives as though nothing is wrong.

Make the best of your talents; don't let DBA control your life.

"Your success depends on what you do yourself, with your own means," - P.T. Barnum. Many famous people have had or do have a medical setback they have to deal with. For example, the lead singer of the band "Something Corporate" was diagnosed with lymphoblastic leukemia in June of 2005. Nick Jonas, of the Jonas brothers, has Type I diabetes. Magic Johnson has AIDS. Look at how successful they are.

Reality Check

You can achieve anything you want. If someone is telling you that you can't do something because of your blood disease, surprise them! Blow them away with how amazing you are at it. I'm not saying it will be easy; in fact, it wouldn't be easy for anyone. If you work hard to get it, the feeling will be indescribable. Remember Miley Cyrus' song "life's a climb, but the view is amazing." Be all you can be, put yourself out there, work hard, do what you want, live your life and everything will fall into place. DBA is not a setback; it's just a reality check. We all need to come together and make the best of our Diamond Blackfan Anemia. Dream... Believe... Achieve... the new acronym for DBA.

Paige's Page will be a regular column written from a teenager's point-of-view. Paige goes to school, she gets blood transfusions, she chelates, she conquers. If you have any encouraging thoughts, quotes, stories or questions, send them to paige@dbafoundation.org, and they may be incorporated into the next DBA Newsletter. Requests for anonymity are respected.

The Twelve Months of DBA

by Betty Lightner

On the first month of treatment, my doctor gave to me...	On the sixth month of treatment my doctor gave to me	And a hemoglobin of nine point three	Eleven hours of Desferal
A hemoglobin of two point three.	Six DBA genes,	Three	Ten gulps of Exjade,
On the second month of treatment,	Five doc referrals,	On the ninth month of treatment,	Nine mg predinose,
my doctor gave to me	Four CaringBridge friends,	my doctor gave to me	Eight donor matches (we wish!),
Two needle pricks,	Three research studies,	Nine mg predinose,	Seven transfusions,
And a hemoglobin of three point three.	Two needle pricks,	Eight donor matches (we wish!),	Six DBA genes,
On the third month of treatment,	And a hemoglobin of seven point three	Seven transfusions,	Five doc referrals,
my doctor gave to me	On the seventh month of treatment	Four CaringBridge friends,	Four CaringBridge friends,
Three research studies,	my doctor gave to me	Three research studies,	Three research studies,
Two needle pricks,	Seven transfusions,	Two needle pricks,	Two needle pricks,
And a hemoglobin of four point three.	Six DBA genes,	And a hemoglobin of ten point three	And a hemoglobin of twelve point three
On the fourth month of treatment,	Five doc referrals,	On the tenth month of treatment,	On the twelfth month of treatment,
my doctor gave to me	Four CaringBridge friends,	my doctor gave to me	my doctor said I'm free...
Four CaringBridge friends,	Three research studies,	Ten gulps of Exjade,	Twelve point three hemoglobin,
Three research studies,	Two needle pricks,	Nine mg predinose,	Eleven hours of Desferal
And a hemoglobin of five point three	And a hemoglobin of eight point three	Eight donor matches (we wish!),	Ten gulps of Exjade,
On the fifth month of treatment,	On the eighth month of treatment,	Seven transfusions,	Nine mg predinose,
my doctor gave to me	my doctor gave to me	Six DBA genes,	Eight donor matches (we wish!),
Five doc referrals,	Eight donor matches (we wish!),	Five doc referrals,	Seven transfusions,
Four CaringBridge friends,	Seven transfusions,	Four CaringBridge friends,	Six DBA genes,
Three research studies,	Five doc referrals,	Three research studies,	Five doc referrals,
Two needle pricks,	Four CaringBridge friends,	Two needle pricks,	Four CaringBridge friends,
And a hemoglobin of six point three	Three research studies,	And a hemoglobin of eleven point three	Three research studies,
	Two needle pricks,	On the eleventh month of treatment,	Two needle pricks,
	Three research studies,	my doctor sent to me	And a happy DBA remission for Bailey!!!
	Two needle pricks,		(hopefully someday)

You are cordially invited to celebrate the publication of

The Match

"Savior Siblings" and One Family's Battle to Heal Their Daughter
Beth Whitehouse



The Match chronicles Stacy and Steve Trebing's quest to cure their daughter by creating a brother who could give her a bone marrow transplant.

"A thought-provoking, extremely well-researched, and deeply personal account of one of the most controversial ethical dilemmas of our time."

—Rachel Simon, author of *Building a Home with My Husband* and *Riding the Bus with My Sister*

"A riveting, vividly written tale of what happens when two powerful forces—parental love and modern science—converge to try to help a very brave child through the deliberate conception of another."

—Liza Mundy, author of *Everything Conceivable: How Assisted Reproduction is Changing Our World* and *Michelle: A Biography*



For more information, visit www.beacon.org/thematch.

Upcoming Events

Thursday, April 8, 7:00 P.M.

Book Revue
313 New York Ave.
Huntington, NY 11743
For information:
www.bookrevue.com
631-271-1442

★ The Trebing family will be in attendance.

Friday, April 30, 8:00 P.M.

Think Coffee
1 Bleecker St.
(Corner of Bowery & Bleecker)
New York, NY 10012
For information:
www.thinkcoffeenyc.com

Thursday, May 6, 7:00 P.M.

Barnes and Noble
3981 U.S. 9
Freehold, NJ 07728
For information:
732-409-2929

Stay updated by connecting with the Diamond Blackfan Anemia Foundation

For comprehensive information related to DBA, please visit our website frequently. On Facebook, become a Fan of our Page and a Member of our Cause. Sign up to follow our tweets on Twitter. Join an email support group on Yahoo.

www.dbafoundation.org

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www.twitter.com/DBAFoundation

blackfan@yahoo.com dba21@yahoo.com



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