OUR HIKE Progress Report

From the blog of Robin Grapa and her mother, Patty Laatsch, as they continue their hike across the country to raise $1,000,000 to help bone marrow disease patients and their families:

Somewhere on Lockhart Basin Rd., Utah – “This was the hardest two-day stretch we’ve had so far. It was tough. This morning we knew we had underestimated how much water we were going to use, so we were already conserving. We started out with only 2 liters each, and we had over 20 miles in the heat to hike. Not cool.

We didn’t stop at all today except for a few short snack, water, and shade breaks. We were really getting nervous about our water shortage when we had about 12 - 15 more miles to go, and almost no water left. It rained this morning in some places, so we came across a puddle on a rock. We scooped it up and filled one of our liter bottles. It was orange, but with a couple of purification tablets, it was gold to us! Mom and I took turns chugging it until it was gone. It didn’t taste too bad -- a little like mud, a little like iodine, and it had a gritty texture to it. But it felt good to drink…”

You can help Robin and Patty in their quest -- not by supplying them with water, regulating the crazy weather, or lightening their load. Robin and Patty need your help to accomplish what they set out to do – raise $1,000,000 so AA&MDSIF can continue to spread the word about bone marrow disease and continue to help patients and their families long after “Our Hike” is over. Meanwhile, Robin and her mom just keep hiking, wishing they could do more.

You are the key:
- Donate to “Our Hike” and ask your friends and family to do the same.
- Find corporate sponsors to help. Do you know of a company that would like to join current sponsors, 4imprint and Celgene? The “Our Hike” web site will remain active for a long time!

“This hike is our attempt to spark an ongoing effort to not only save lives, but give hope, inspiration, and maybe help a newly diagnosed victim of bone marrow disease by letting them know they aren’t alone,” says Robin. “So while we’re in these desolate areas of the country, we will do what we can and push ourselves toward the Pacific Ocean. But we need your help to reach out to more people. We won’t give up until we reach that $1 million, and we know you won’t, either. The ‘Our Hike’ family of supporters, friends, family, and patients is a tool that we can all use to make a tremendous difference.”

Read more about Robin and Patty’s cross-country trek as they walk to raise $1 million to benefit bone marrow disease support, education and research. Visit their web site (use the link from www.aamds.org) and track their progress as they head toward the Pacific Ocean. The hikers are due to arrive at their goal on the California Coast the last week of October. To donate or for help contacting corporations or national media, contact AA&MDSIF at 800-747-2830 or visit www.aamds.org.
From the President...

As to diseases, make a habit of two things – to help, or at least to do no harm. The art has three factors, the disease, the patient, the physician. The physician is the servant of the art. The patient must co-operate with the physician in combating the disease.

-Hippocrates

Healing is an art form. Does that sound like New Age groupie talk? It’s easy to forget that, even with all of our advanced technology and the dazzling brilliance of computers, we are still completely dependent upon the human element in our health care. While we wish that healing was an exact science, alas it is not. There is an art to making educated medical decisions that relies on the human element - doctors, nurses, physician assistants, and other healthcare professionals.

It is as true today as in 380bc, when Hippocrates sat in his physician’s waiting room, that an important part of the doctor’s decision-making is input from the patient. Patients cannot just sit idly by and watch what is happening to them; they have to be an integral part of the process. Patients have a major job to do (in addition to being sick): obtain information, gain understanding, weigh the pros and cons, assess risks and benefits, follow instructions, give feedback, and think positive. Then, and only then, can patients fully co-operate with their physicians in making decisions that are right for them and participate in the art of their own healing.

This is why part of the AA&MDSIF’s mission is to give patients the information and support they need to be able to work well with their doctors. We do this by providing free services, connections to other patients and families, access to various medical resources, a staff of information specialists who work as patients personal medical researchers, booklets written by leading medical experts, constantly updated medical information, and personal answers to your questions from the world-renown doctors on our Medical Advisory Board. Make sure you are using the full extent of our staff and resources. Call us today and put us to work for you!

- Marilyn Baker, M.S. President & Editor

Attention Medical Researchers...

Applications are now being accepted for our AA&MDSIF funded Research Awards. The AA&MDSIF is interested in funding medical research relating ONLY to the prevention and treatment of acquired bone marrow diseases - aplastic anemia, myelodysplastic syndromes, and paroxysmal nocturnal hemoglobinuria. Application forms can be downloaded: www.aamds.org.

Prescription Drug Coverage...

Unfortunately many of the co-pay assistance programs for pharmaceuticals run out of funds. Call them regularly to see if they have funding: HealthWell Foundation (800) 675-8416 www.healthwellfoundation.org.

Luckily, the Patient Access Network and the Chronic Disease Fund are still accepting applications:

- Patient Access Network: (886) 316-7263 www.patientaccessnetwork.org

Call for Entry – Children’s Artwork...

We are in need of artwork from children under the age of 18 who have bone marrow disease. The pieces will be judged and used for various projects such as the Children’s Support Network on our website. For more information go to www.aamds.org or contact Jennifer Krammes, krammes@aamds.org (800) 747-2820.

Be the First to Know...

Sign up for the AA&MDSIF E-Bulletin to receive regular updated medical research updates on bone marrow disease and treatments. Patients, caregivers, nurses, and doctors should send their enrollment request to: finne@aamds.org.

Clinical Trials...

An updated version of our brochure, Clinical Trials for Patients with Bone Marrow Diseases: Basic Explanations, has just been published. For information about clinical trials, contact Beth Bradley at bradley@aamds.org (800) 747-2820.

Holiday Cards that Help Fight Bone Marrow Disease...

A variety of lovely cards wishing lyrical messages of the season are now available at www.aamds.org or by calling (248) 624-1394. These special cards will let family and friends know that your holiday greeting is also helping to fight bone marrow disease!
Meet the Officers of the AA&MDSIF Board of Directors

Neil Horikoshi, Chairman of the Board

“People wonder why I’m so committed to the foundation. I tell them there is a passion and a dedication here that is impressive.” Neil credits the foundation with helping him get through his anti-thymocyte globulin (ATG) treatment for aplastic anemia and now he wants to give back by serving the organization. A high-ranking international executive with IBM, Neil brings corporate knowledge and a passion for finding a cure to AA&MDSIF. He is committed to advocating for greater research and awareness that will ultimately lead to a cure.

Neil’s vision is for the Foundation to be instrumental in finding the root causes and cures for bone marrow diseases. He feels we can do that by reaching out to even more patients, increasing awareness of the organization’s mission, and raising more money to support innovative research. “Medical professionals, legislators, corporations, foundations and society in general do not know enough about these diseases,” says Neil. “If they knew, I know they would support our efforts.”

Tamir Orbach, Vice-Chairman of the Board

Tamir received a bone marrow transplant in 2004, and today his PNH is no longer a part of his life. Nevertheless, he is still actively engaged in PNH issues. A Microsoft employee, he would like to see more public awareness of AA&MDSIF and the services and resources it offers to patients, caregivers and medical professionals. “Doctors and nurses often don’t even know much about bone marrow diseases,” says Tamir from personal experience. “We need to find a way to reduce the risks and help patients make informed decisions.”

The future holds much for Tamir – especially with the birth of his daughter, Allison – and he feels it holds much for us, too. He wants to help AA&MDSIF find new ways for patients to connect with each other, with experienced doctors and with drug companies for trials. “We need to make sure doctors understand that they should refer patients to medical specialists in bone marrow diseases – and to us for support.”

Ruth Cuadra, Secretary

“AA&MDSIF was the lifeblood of my recovery,” says Ruth Cuadra. The information she got when first diagnosed helped her talk to her doctors and gave her hope. It was important to know that she was not alone and that other patients were out there. Ruth was diagnosed with aplastic anemia in 1996 and had limited success with ATG treatment – until she was diagnosed with myelodysplastic syndromes (MDS) two years later. Luckily, an unrelated match made it possible for her to have a bone marrow transplant, and she has been relatively healthy since.

Ruth advocates strongly for awareness of bone marrow diseases in two vulnerable populations: seniors and youth. The MDS population tends to be older men and women – a population that will be growing over the next couple of decades as the baby boomers age. Aplastic anemia often affects younger patients and outreach to pediatricians and college campuses health groups can help.

Tony Sanfilippo, Treasurer

A professional in the world of finance for over 30 years, Tony came to us during the illness of his daughter, Rachel. An accomplished gymnast, her successful ATG treatment has kept her medication-free for over 8 years. Tony’s pride in his daughter and his concern for other children and their families led him to become involved.

Tony would like to see increased awareness of bone marrow diseases, and he wants AA&MDSIF to have the long-term financial strength to continue research grants and to find a cure. “Sadly, bone marrow diseases are orphans in many ways,” says Tony. “It’s difficult to get as much recognition and funding support as some others, such a cancer, on a national level.”

Tony notes that the board members all have different perspectives on bone marrow diseases; there are patients and caregivers with experience in AA, MDS and PNH. Many have maintained an interest level and attachment to the organization even after the diseases are out of their lives. “Feelings for AA&MDS and its work have kept us involved,” says Tony. “The board members want to continue to be part of the success and growth of the organization.”
BYRNE - AMUSO RESEARCH AWARD
“Having a research fund named for our son Jack *(pictured here)* is a great honor,” say John and Kristen Byrne. “It gives new meaning to his courageous battle with aplastic anemia.” The Jack Byrne Research Fund partnered with the Marissa Marie Amuso Research Fund to provide a grant to Dr. Lisa Minter of the University of Massachusetts-Amherst to study whether gamma-secretase inhibitors may provide additional treatment options for patients with aplastic anemia and lessen the severity of the disease. Marissa’s mom, Kim, works tirelessly to raise money for the research project named for her daughter. “I’m one of the lucky ones because I still have my baby here with me,” says Kim.

EARL J. GOLDBERG RESEARCH AWARD
For more than 25 years, the friends of Allen and Harriet Goldberg raised money to help patients and families with aplastic anemia in honor of their son, Earl, who passed away at the age of 14. Today some of the $171,000 they raised is supporting Dr. Seth Corey of UT-MD Anderson Cancer Center in his study of the effects of an enzyme on controlling the demise of blood cells. “I became interested in hematology as a resident at St. Louis Children’s Hospital in 1983 when I took care of a 9-year-old girl with aplastic anemia.” Dr. Corey explains. “There has been tremendous progress since then, but it’s still not enough.” He feels that what we will learn in this study, knowing how to restore the balance of blood cells, can improve therapies for patients.

HOLLY CATALDO RESEARCH AWARD
In 1999 when Holly Cataldo was diagnosed with aplastic anemia, she decided to channel her fear into raising money to support research by creating a “Car Cruise.” Sadly, Holly lost her battle and today her sister, Christina Beranek, and her family continue the tradition that Holly started, holding a Car Cruise each Labor Day weekend. Holly’s legacy is supporting the work of Dr. Hinh Ly of Emory University. Dr. Ly is studying the biology of a unique DNA sequence, called telomere, which acts as a ruler to measure the life span of every cell in our body. “This study will provide us with a better understanding of the possible genetic factors contributing to AA and MDS,” says Dr. Ly. “This may lead to the development of effective therapies against these diseases.”

PNH RESEARCH and SUPPORT FOUNDATION AWARD
As you can read on page 7, the PNH Research Foundation has gone to great lengths -- two miles around NYC’s Riverbend to be exact -- to raise money to fund research. This year’s funding will support Dr. Lukasz Gondek at the Cleveland Clinic Taussig Cancer Center. Using gene microarrays and a large collection of DNA from patients, Dr. Gondek will study inherited and acquired genetic defects that result in bone marrow failure diseases. “Our hope is to create diagnostic tests that may predict individual risk and identify those who could best benefit from specific types of therapy,” says Dr. Gondek.

LINDSAY MINELLI RESEARCH AWARD
Also at the Cleveland Clinic Taussig Cancer Center, Dr. Christine O’Keefe is studying genome stability in MDS. Like Dr. Gondek’s work, the study will seek to identify markers to further the diagnosis and therapy for MDS. Her work is being funded by the Lindsay Minelli Research Study. “It all happened so fast,” says John Minelli of the death of his daughter. Within four months of her MDS diagnosis, Lindsay was gone. John decided to honor her memory by providing the funds for a research grant. “If this research study does some small part to help find a cure, other families will be spared the pain we have suffered,” says John. “This is for Lindsay.”

This is a particularly meaningful and loving tribute to keep the memories of loved ones alive in the hearts of so many. The establishment of a grant can spark community involvement in fundraising, and at the same time it can raise awareness and understanding of bone marrow diseases. You can support a life-saving research grant, too. Call us today at 800-747-2820 or go to www.aamds.org/donate and click on the “Types of Donations” link to learn more about the Research Grant Program. 100% of the funds raised will be directed to medical research to find new effective treatments for bone marrow disease, and ultimately - a cure.
What Your Donation to Research is Doing!

AA&MDSIF Funds Research at the Cleveland Cancer Clinic

Jaroslaw P. Maciejewski MD, PhD
Taussig Cancer Center, Cleveland Cancer Clinic

The human genome project decoded all of the genes in the human body. Although we all carry the same set of genes, the differences between humans, including their individual susceptibility to diseases, are due to discrete differences (referred as to polymorphisms) in the structure of thousands of genes. This multitude of discrete variants of each gene combined in a complex relationship that may determine the risk of bone marrow disorders such as paroxysmal nocturnal hemoglobinuria (PNH), aplastic anemia (AA) or myelodysplastic syndromes (MDS). Of course environmental triggers are also required for the disease to develop; therefore, having a susceptibility variant of a gene does not necessarily mean that it will develop. The diagram below explains the relationship between genetic and environmental factors. Conversely, very strong external factors may override genetic disease resistance.

![Genetics and External Factors in Disease](image)

For years we have studied whether individual gene variants (also called polymorphisms) are connected to the risk of PNH, AA and MDS. However, these studies were very inefficient as our view was only limited to a selection of singular polymorphisms out of the pool of millions possible. A recently developed technology, referred as to “SNP microarray, gene chip or genetic scan”, makes such studies more efficient allowing for the analysis of hundreds of thousands of gene variants at once.

The previous funding from Aplastic Anemia & MDS International Foundation enabled us to test these technologies and we can now apply them to detect chromosomal changes in a much more precise fashion than it is currently possible using routine chromosomal tests. Consequently, we can better distinguish bone marrow disease and its individual forms and better predict prognosis. However, there are new applications of SNP microarrays. For example, we would like to utilize this technology to study extremely large numbers of gene variants (500 000 per patient) in many patients with PNH, AA, MDS to identify genetic risk factor constellations that would explain why certain individuals are affected by these diseases. Moreover, a very similar principle of the genetic scan can be applied to look for possible viruses that could trigger disease. This technique is called a Viral Scan. Unlike in the past when we studied whether selected viruses are implicated in patients, Viral Scan allows for testing for the presence of thousands of viruses at once, including those that have not been previously described as causing human disease. We hope that systematic use of such technologies will help solve the enigma of what genetic factors constitute the risk for bone marrow disease and identify the viruses that could possibly trigger AA, MDS, and PNH.

This research was funded by the PNH – Pursuing New Hope Award created by a loving father as a Valentines Day Gift through the Aplastic Anemia & MDS International Foundation, Inc.
Patience, Trust and Hope

When Sister Stephana Toomey was diagnosed with MDS in 1992, she knew it was a serious illness, but it wasn’t until her platelet count plunged dramatically in 1993 that she became afraid. Unfortunately the news was not good and she was told she had less than two years to live. In response to this dire prediction her hematologist told her, “Be patient.” That advice has stood her in good stead since then. Between her hematologist and her internist, who acts as a liaison between her and other doctors, she has learned to be patient, to take things easy, and to trust. “If you have a physician you trust, stay with him,” says Sister Stephana. “I’m grateful I did. They have both been wonderful in their own individual ways.”

Sister Stephana Toomey is a member of the Dominican Order, and this year, at the young age of 75, is celebrating her Jubilee (50th Anniversary.) Today, fourteen years after her diagnosis, she is going strong and incredibly busy. As a liturgical artist and a consultant to churches of every denomination, she often is stressed with deadlines and the challenges of her craft but she takes it all in the stride of life, “If anything, I’ve done some of my best work since that health crisis.” Her nonprofit organization, Efhirasto Studio, (Efharisto is Greek for “thank you”) is a design studio that partners with congregations and architects to design churches, alters, stained-glass windows and other liturgical works. Sister Stephana’s inspiring work can be seen on her web site: www.efharisto.org.

Victory!

48 States Down, 2 to Go!

Many people might be discouraged by the twin diagnoses of MDS and PNH, but Leonard Peterson decided to make the most of his life and not allow his illnesses to get him down. “It might sound like a cliché, but being close to death truly helped me to appreciate the fact that every day is precious,” says Leonard. He retired from his job and began thinking about his life. He assessed where he’d been and where he was going – literally. Making a list, he realized he had visited almost every state in the Union over the years. That “almost” was a sticking point for him, so he set his goal: to visit all 50 states in the U.S.A.

Leonard and Sandy just returned from their latest adventure – a trip to Wyoming, Idaho, Montana, and Washington. They spent a lot of time in Yellowstone and the Grand Teton, enjoying the beautiful scenery and the chance to see buffalo, elk, eagles and other wild animals. He has only two states left on his list: North Dakota and South Dakota, where he will visit next year. At 61 years of age, Leonard is loving life and looking forward to checking off the last two states on his list. When asked what he will do for an encore, he doesn’t hesitate. “We’ve always wanted to go to Spain and Israel,” says Leonard. “We’re already making plans for those trips.” In the meantime, Leonard and Sandy are hitting the road again next month to attend a convention in California. There’s no pinning Leonard Peterson down!

Leonard and Sandy passing through Montana on their travels.
NYC Walk-a-Thon Raises $38,000 for PNH Research

Dr. Sara Higgins, PNH patient and organizer of the PNH Research and Support Foundation, was delighted by the turn out at her first PNH WALK-A-THON. She expected 30 walkers to take the challenge and walk 2 miles at New York City’s Riverbend Park. She was in for a surprise. “It was amazing to see 150 people show up!” exclaims Sara.

The PNH WALK-A-THON was in memory of Bob Bisher, a PNH patient and member of the PNH online support group. The event took place on Sunday, September 10th and raised over $38,000 for PNH medical research through the Aplastic Anemia & MDS International Foundation.

Email announcements to hematologists in the New York area brought in a number of hematologists, nurses and their patients who joined in the fun. And Sara was delightfully surprised again when she saw a busload of supporters from walk sponsor Alexion Pharmaceuticals pull up with employees and family members all the way from Connecticut to help make this walk such a success.

Sara says that people along the walk route stopped walkers wearing the distinctive walk t-shirts to ask about the cause, many making donations right on the spot. Bob’s widow, Meghan, was also thrilled with the results of the walk. “Bob would have been so incredibly proud!” says Meghan. Sara is already planning next year’s walk – the 2nd Annual of many walks to come. She hopes to have twice as many walkers and raise twice as much money to support PNH Research.

PNH WALK-A-THON participants gather in NYC
Immunizations and Flu Shots

There is uncertainty among experts as to whether or not aplastic anemia patients should get a flu shot. Some doctors advise patients not to get flu shots as this may worsen blood counts, perhaps by stimulating the immune system. However, other doctors feel the risk of the flu’s illness outweighs potential harm from the flu shot. There is minimal real data on this subject. The decision to get a flu shot should be discussed with the patient’s hematologist to determine individual risks and benefits. In general, live vaccines should not be administered to individuals who are immunosuppressed.

The following discussion, originally published in the Fall 1999 newsletter, outlines some of the concerns about immunizations and flu shots:

Generally, patients with active disease should not get immunization shots nor flu shots since this may cause worsening of blood counts. Patients should not be exposed to another family member who has received shots for 2-3 weeks if this is practical.

There is little risk of deterioration of blood counts from using killed vaccines. Tetanus falls into this category. However, one should remember that protection from tetanus lasts at least ten years and longer in most individuals. Therefore, unless a patient has suffered a severe injury, there is not an urgent need for most people to receive an other tetanus immunization.

A pneumococcal vaccine is a killed vaccine and can be given to patients who are high risk for infection with these organisms.

The risk of acquiring polio (even mild infection) from a vaccinated sibling is extremely low and the period of potential isolation from the vaccinated sibling is potentially long. Possibly the best choice is to vaccinate the sibling. A second choice would be to have the patient or the sibling live elsewhere for a month. The least desirable option would be not to vaccinate either patient.