Dear Support Group News Subscriber:

Welcome to the first issue of the PNH Support Group News, the informative newsletter published to keep you informed of the latest news. In the few years I have had PNH, I have found there is not a lot of information on this rare disease. I had a lot of questions and concerns when first diagnosed. In fact, 4 years later, I am still learning daily about the disease. I was happy in 1999 to establish an E-mail support group for PNH suffers, family and friends. I am now taking the second step in providing support to fellow PNH patients with this newsletter. My hope is that together we can spread more awareness and focus on PNH, not only to the medical field, but to others living daily with PNH. I welcome you all to share your ideas and thoughts for further support and help with providing a useful newsletter. Please send email to PNHsupport@AOL.com.

The PNH Support Group

http://groups.yahoo.com/group/PNHdisease

The PNH Email Support Group provides members access to useful information 24 hours a day, 365 days a year.

Founded in December 1999, it has been an enormous support for people afflicted with PNH, and their family and friends. Every month the group has grown, proving that there is more need for more information about PNH. The e-mail support group is hosted by Yahoo Groups.

To join go to web page:
http://groups.yahoo.com/group/PNHdisease
OR contact Rebecca Gaskin by e-mail at Rebecca270@AOL.com.
Paroxysmal nocturnal hemoglobinuria (PNH) is a rare disorder, and it is not a cancer. PNH is a form of anemia, which means that the red cells circulating in the blood are decreased. The most prominent problem is excessive destruction of red cells (the technical term is hemolysis: thus, PNH is a hemolytic anemia); but because hemolysis takes place in the blood stream, the red cell contents – mainly hemoglobin – pour out in the urine (hemoglobinuria). This is the reason for the dark urine, which sometimes is almost black. In many patients the early morning urine tends to be darker, hence the word nocturnal (meaning ‘during the night’): however, this is not a rule and it is of no importance. In fact, hemolysis in PNH goes on all the time, but its intensity varies, and it can become suddenly much worse (paroxysmal) when there is an infection, or after strenuous physical effort, or for other reasons, not all of them known. In addition, the bone marrow is not always able to step up production of red cells sufficiently to keep up with their destruction: thus, there is an element of bone marrow failure. When this is severe, there may be a decrease not just of red cells, but also of white cells (called leukopenia or neutropenia) and/or of platelets (called thrombocytopenia). When all 3 cell types are decreased the technical term is pancytopenia: in such cases PNH comes close to a condition in which bone marrow failure is the primary factor, called aplastic anemia (AA). Very rarely PNH can transform into leukemia: but it is not leukemia, and it is not cancer.

PNH is also referred to as a chronic hemolytic anemia, because it can go on for a long time. Although the dark urine may be perceived as unpleasant, fortunately it does not by itself cause any harm to the kidneys or to any other body system. PNH is more complex than AA. The main additional problem is that about one-half of the patients PNH sooner or later may develop venous thrombosis (clots within the veins), particularly in the area of the liver, the spleen, the gut. However, most of the time the vigorous activity of the PNH bone marrow is an advantage in comparison to AA. That is why PNH has been also referred to as ‘a blessing in disguise’.

In some cases PNH can be treated by bone marrow transplantation (BMT) from a suitably matched brother or sister, when available. In other cases there may be indication for the use of anti-thymocyte globulin (ATG). In all cases it is important to provide good support by periodic clinic visits in a hospital where there is experience with PNH. Support may include blood transfusion and long-term anti-coagulants may be also indicated.

Lastly, it is important to know that ‘living with PNH’ is possible, and in some cases, after many years, spontaneous cure may take place.

Encouragement

"The fact that many patients with PNH live many, many years (and may have a normal life span) means that all patients need to conduct themselves as if they were going to do the same."

From: What is PNH? A Guide for Patients, Wendell F. Rosse, M.D. Retired, Duke University Medical Center
The Aplastic Anemia and MDS International Foundation (AA&MDSIF) has graciously agreed to help with the process of making PNH Research possible. It is with great honor that I name the first PNH Support research fund after our dear friend, Deb Valcik.

We came to know Deb in our PNH Email Support group in December 1999, shortly after the support group was established. Deb was diagnosed with PNH in September of 1999, but had been sick with Anemia for 5 years prior to her diagnoses. She continually had low counts and felt terrible. In March of 2000, Deb was preparing for a Bone Marrow Transplant, but at the last moment was treated with ATG (Antithymocyte Globulin). During the next year, unfortunately in her case, the ATG treatment was unsuccessful for Deb. In February 2001, Deb started the process for her Bone Marrow Transplant, with no fear, but the desire to be cured of PNH. Complications arose during her Transplant, and from there Deb experienced a battle beyond belief. Her husband, Steve, faithfully wrote and kept us updated. Deb and Steve, supported and comforted by their faith, were an inspiration to us all. During the darkest days, Steve would write to share the highs and lows Deb had endured. Steve’s powerful love of his wife, and enormous faith never faltered. His written words pierced our hearts tenderly and drew us together, each of us anticipating his next update. We all prayed, Deb beat many odds, but over all succumbed, passing away April 7, 2001 at age 40.

The Deb Valcik PNH Research Fund

“It is with great honor that I name the first PNH Support research fund after our dear friend, Deb Valcik”

– Rebecca Gaskin

The AA&MDSIF is a 501© (3) nonprofit organization as recognized by the Internal Revenue Service. A named Research Fund is $30,000 a year for two years totaling $60,000. One hundred percent (100%) of all money donated to The Deb Valcik PNH Research Award will go to medical PNH research – not one penny is taken out for any type of administrative expenses. For unspecified donations, the AA&MDSIF is proud of their efficiency, spending only 4.7% on administrative costs and 1.5% on fundraising expenses. Please compare their record with other charities when gift giving. When the goal has been met, the AA&MDSIF Medical Board will review applications from researchers all over the world. The Medical Board is composed of distinguished experts, who will select an applicant who is deemed to be worthy of funding.

All of us afflicted with PNH, and family members of those suffering, would love to see a cure for PNH found. This disease is disabling to some, fatal in others. With PNH being a rarity, an unknown disease to many, it is like a shadow in many lives, dark but always present. Now we have the opportunity to help find a cure. I thank all of you in advance for any help you are willing to provide.

Encourage someone to Register to donate marrow or Stem Cells.
When you become a donor through the National Marrow Donor Program (NMDP), you are participating in an effort to give patients with PNH, AA, MDS and other life-threatening blood diseases a second chance at life.

Call NMDP at 1-800-MARROW2
always look forward to spring. It is the time when life is reborn and the darkness of winter becomes a memory. As I was getting ready for my day that April of 1992, all I could think about was the anticipation of the Easter weekend. After school that day, I had a routine doctor’s appointment. In spite of my schedule and all the running around I was doing, I felt fine. That doctor’s appointment would change the rest of my life.

At the doctor’s office I spoke to a nurse, who agreed with me. The doctor, however, agreed with my father and ordered all types of lab work. I was frightened. The next day, as I awakened, I heard my Mom on the phone, talking to the doctor’s office. The doctor wanted to admit me to the hospital. Something in my blood work was not right. My platelets were at 20,000 (normal platelet counts vary from 150,000 to 450,000) and I could be susceptible to spontaneous bleeding. Platelets? Counts? Bleeding? I had been frightened before, now I was terrified.

When I got to the hospital, a new doctor (who became a close friend over the coming years) introduced himself. He explained that they were going to have to do a bone marrow biopsy, to rule out Leukemia. After hours of waiting, the biopsy came back and was normal. So what was wrong with me?

After a weekend in the hospital, I was told that the possibilities had narrowed to two different diagnoses. The first being a vitamin B-12 deficiency, which is easy to treat. The second diagnosis was a bit more complicated.

Four days later, we learned that my vitamin B-12 level was normal. It was not a vitamin deficiency.

The doctor explained that during his residency at Duke University he had studied a rare blood condition called Paroxysmal Nocturnal Hemoglobinuria, referred to as PNH. He decided to send my blood work to Duke University to be studied.

The test came back positive for PNH. What did this all mean? We were so confused. My life felt shattered.

The first step in tackling my PNH was to be put on medications that would help my blood counts. I was put on Danazol, Prednisone, and Folic Acid. The next step was to get tested for sibling compatibility for a possible future Bone Marrow Transplant. My sister, Kathleen, was a perfect match. My doctor thought that the bone marrow transplant should be done as soon as possible, since PNH has a chance of turning into Leukemia.

My doctor was all set to refer me to the University of Pennsylvania hospital for a Bone Marrow Transplant in the fall of 1993. All he waited for was to get a second opinion from his mentor, Dr. Rosse at Duke University.

When I walked into the doctor’s office a couple of days later, he was in good spirits and told us that Dr. Rosse did not seem to think the BMT was necessary. It was the first power of prayer that I would experience!

Living with PNH was not too bad for me. My parents and I made summer vacations out of visits to Duke University. Each year we were more and more educated about the research being done. Each year, we would leave being relieved that the Bone Marrow Transplant was still on the back burner. I was going to school at Penn State at the University Park campus. I had my moments with PNH, but I was always in good hands. I adapted to a medical condition and didn’t let it rule my life.

Exercise has always played an important role in my life. After receiving a diagnosis of PNH ten years ago, I continued my regimen of running and aerobic exercise classes, with the support and approval of retired PNH Specialist, Dr. Wendel Rosse. Over the past two years, particularly during the Fall and Winter months, I had begun noticing a decrease in my overall energy level. This, coupled with increased hemolysis, had left a scant amount of vigor for my customary exercise regimen. Fortunately, this past October, a dear friend introduced me to Hatha Yoga, which almost instantly...
I successfully made it through student teaching with perfect attendance and graduated in the spring of 1994. I started to substitute for a while and did not land a full time teaching position until January of 1996. PNH was just an ordinary, every day thing for me. I would take my meds and get my blood tested. I never missed a day of school, and life was good!

I was coming home from a friend’s house right after Christmas of 1997, and I was a bit itchy, but thought it was due to wearing new clothes. The next day, I woke up and could not get out of bed. I was weak and had no clue of what was wrong. My Mom immediately called my hematologist and we made an afternoon appointment. I was tested for everything and all the tests came back negative. I went home, feeling weak, so I rested. When I was not better in a week, I went back to see my Hematologist. He was on vacation, so his associate filled in. He examined me and could not find what was causing me to be so weak. As a last resort, he decided to get a CBC on me. The nurse ran my blood work twice because she thought there was something wrong with the machine. My hemoglobin was 3 and I was conscious! I was taken to the Emergency Room and admitted. Because I was conscious, the admitting nurse did not see an urgency to get my blood. My doctor that night, in a rage, ran to get the blood himself. He knew I didn’t have any time. After a weeks stay, I was discharged, but still weak.

I came home and was taking it easy for a couple of weeks. I had a friend of mine take over my class. One day, I made an appointment with my friend to go over lesson plans. I was feeling so much better, that I thought that I wanted to work part-time in the afternoons during the week. I had a headache that day, which just would not go away. It was great getting out and back into things. After a tiring day, I still had a headache. It was not your typical headache. I tried to shake it off and chalked it up as doing too much, too soon. The next morning I awoke with pins and needles in my hand. By 7 that night I was not making any sense when I would talk to my parents. By that time I was getting ready for bed, I had to think about how I would turn off the faucet after brushing my teeth.

The next morning we were in the hematologist office. He sent me downstairs to a neurologist, who sent me down to another floor for an MRI. After waiting all day, I was admitted to the hospital and immediately put on heparin. I had had a stroke, which, was later determined to be caused by PNH. I spent several frustrating days in the hospital. The only visitor that I had was my sister, who was there in case I needed to do the bone marrow transplant. Later in the week, I was transferred to the hospital at the University of Pennsylvania. I was discharged a few days later. I had lost control of my right hand, but through therapy and the grace of God, I totally recovered.

I went on with my life. I was able to go back to teaching in the fall of 1998 and life with PNH was good again. At the end of the school year, I decided to change jobs. There were a lot of reasons, personal as well as medical that determined my decision. I got a job as an administrative assistant at a recruiting firm. I adjusted to my new schedule and things were stable. But they were not as good as they had been. I was on large doses of Prednisone and had more episodes of hemolysis than before.

In June of 1999, we were saddened by the news from Duke. Dr. Rosse was going to retire and not be our PNH research source anymore. Not only that, but after seeing Dr. Rosse for the last time, he told me that he felt that my meds were not working enough to keep me at a good count. Furthermore, he thought it was time to consider the BMT.

“**My doctor that night, in a rage, ran to get the blood himself. He knew I didn’t have any time.**”

Continued in next issue . . .
In the several following issues of PNH Support News, we will profile doctors in the PNH news. Many have already replied with the interviews. A big thank you, Doctors, for your time and help with PNH, and supporting patients

Dr. David J. Araten is currently working in the PNH field at Memorial-Sloan Kettering Cancer Center in New York, New York. He has a MD from Harvard Medical School, 1991. I want to sincerely thank Dr. Araten for his willingness to participate in the PNH Physician Profile.

Q: How many years have you been in practice?
A: 10
Q: How many years have you been treating PNH?
A: 6
Q: What prompted you to make PNH your medical specialty?
A: My interest in PNH dates back to patients that I cared for and research projects during my training.
Q: How many PNH patients have you seen in career?
A: 44
Q: Are you currently active in the research aspect of PNH and how long have you been researching PNH?
A: Yes, for 6 years.
Q: In what ways are you and your hospital researching PNH currently?
A: Clinical research on long-term follow-up of PNH patients and risk factors for thrombosis.
Q: In what ways can PNH patients help you further your current research?
A: Our clinical practice has been very helpful in generating a database for clinical research and samples for laboratory analysis.
Q: Where do you see treatment and research going in the future?
A: Cure of PNH patients will benefit in the future from (1) general improvements in supportive medical care (2) improvements in bone marrow transplantation, and (3) our understanding of the pathophysiology of PNH. From our understanding of the relationship between PNH and aplastic anemia, we now know that low blood counts in patients with PNH may be due to an autoimmune process and can respond to immunosuppression. Future progress is likely to come from a greater understanding of the causes of and risk factors for thrombosis in patients with PNH, from new anti-coagulants, and better radiologic imaging.
Q: Have you witnessed a complete remission and how did it present itself?
A: A young woman had been treated with ATG and cyclosporin, developed a PNH cell population, which was practically undetectable (<1%) 11 years later. I was asked by a colleague to analyze a sample by flow cytometry from one of his patients with a long-standing history of PNH; at the time the sample was sent the PNH clone was undetectable. In a third patient who came to see me for a consultation, there was a history of a positive HAM test performed overseas about 5 years prior but no detectable PNH population by flow cytometry when I saw her. A normal lactate dehydrogenase (LDH) level in a patient with a history of hemolytic PNH is sometimes a clue that the PNH clone has regressed. This can be confirmed by flow cytometry.
Q: What type of treatment/management do you prefer most, corticosteroid therapy or Blood transfusion therapy?
A: I do not start patients with PNH on corticosteroids as a treatment for hemolysis or anemia. If a patient with PNH comes to me having already started on steroids, I review their history carefully with them and try to establish if there is reason to believe that the steroids have helped them or if there is another reason why they should be taking steroids. In general, though, I recommend tapering steroids, given their long-term complications, several of which I have seen in patients with PNH. I recommend transfusions of irradiated, filtered blood that is “ABO type specific” (i.e., from a type AB donor for a type AB patient, etc.) based on the patient’s symptoms and the hemoglobin/hematocrit values. In my experience, transfusions are well tolerated and improve not only the anemia, but also seem to have a beneficial effect on the degree of hemolysis.
Q: Have you published any articles in medical Journals on PNH?
A: TITLES:
- Clonal Populations of Hematopoietic Cells with Paroxysmal Nocturnal Hemoglobinuria Genotype and Phenotype are Present in Normal Individuals.
- Allogeneic bone marrow transplantation for paroxysmal nocturnal hemoglobinuria.
- Abnormal T-cell repertoire is consistent with immune process underlying the pathogenesis of paroxysmal nocturnal hemoglobinuria.
- Cyto genetic and Morphologic Abnormalities in Paroxysmal Nocturnal Hemoglobinuria
- Association of clonal T-LGL disease and PNH: further evidence for a pathogenetic link between T cells, aplastic anemia, and PNH.
- Abstracts: The fate of PNH clones over time.

For a complete list of Dr. Araten’s Medical Articles, please Email PNHsupport@aol.com, or write to PNH Support c/o R. Gaskin 1090 Cherylee Drive, South, Salem, Oregon 97302
The 2001 Patient-Family Conference hosted by the AA&MDSIF was held in St. Louis, Missouri on July 27, 2001 - July 29, 2001 at the St. Louis Airport Marriott Hotel.

My desire to attend the Conference came when I read that Dr. Lucio Luzzatto was going to speak on Non-Transplant Treatments for AA and PNH. Dr. Luzzatto has over 30 years experience in the PNH research field and treating of PNH patients, and he happens to be one of the PNH specialists I have seen for my disease. Since I don’t have a sibling for a Bone Marrow Transplant (BMT), I knew this would be a good Conference to attend.

This was my first time attending the AA&MDSIF Annual Conference, and I found it to be very informative, and a wonderful opportunity to meet others with PNH and the related diseases of Aplastic Anemia (AA) and Myelodysplastic Syndromes (MDS).

As a PNH Patient I sat in on the sessions that I felt most applied to my treatment of PNH and the treatment of those I correspond with who have PNH. The sessions I found most beneficial were the Iron Overload Management by Dr. Eric Nisbet-Brown, Non Transplant Treatments for AA and PNH by Dr. Luzzatto, and Transplants for AA by Dr. Richard Champlin.

Although Iron Chelation is rarely needed in PNH cases, it is still something that may occur after many years of transfusions. Most PNH patients loose a lot of iron through their Hemolysis, and additionally so for women, who loose more iron with the monthly menses. But for those living with PNH for 20 plus years and have been transfusion dependent, the need for Iron Chelation may arise.

For those with PNH, and a BMT is not possible, there are a few choices of treatment and management. ATG (anti-thymocyte globulin) is standard treatment for PNH and AA because it helps to raise the White and Platelet Cells, but it does not raise the Red Cells. Red Cell Transfusions can manage anemia; Platelet Transfusions can manage Platelets. For those who have had Thrombosis with PNH, Anticoagulant therapy must be used. Anticoagulant therapy is potentially dangerous to those who have low Platelets due to the risk of bleeding. Currently there is nothing to stop Hemolysis in PNH. Prednisone is sometimes used in PNH, but there are no trials proving it stops Hemolysis. The most radical treatment for PNH would be a BMT. This option is most affective when one has a sibling match.

I am very optimistic about 2002 Family and Patient Conference. During the Conference, I spoke to Marilyn Baker, Executive Director of the AA&MDSIF, and she told me their foundation would put more focus on PNH in 2002.

Information about the Aplastic Anemia & MDS International Foundation can be found at the web site at www.aamds.org or call 1-800-747-2820.

Drug Warning: Neupogen Counterfeit

Amgen Inc. Has recently become aware of the existence in the U.S.A. of a counterfeit drug product labeled as Neupogen 300 mcg vials in ten-pack boxes. In cooperation with the U.S. Food and Drug Administration (FDA), Amgen is informing patients, physicians, pharmacies, and wholesalers about this serious health risk.

Please go to web page: http://www.amgen.com/corporate/AmgenNews.html, where you can view photos and details to determine if your Neupogen is counterfeit.

Many people with PNH are dependent on Red Cells Transfusions or Platelet Transfusions. Encourage a friend of family member to donate blood products today! Call the American Red Cross for more information: 1-800-GIVE LIFE
Paroxysmal Nocturnal Hemoglobinuria Support News is published with the intentions of supporting those afflicted: from patients to family and friends. PNH Support News also is an attempt to raise the level of awareness of PNH and its various treatments by sharing information from multiple sources, including patients, doctors and researchers.

**Web Pages:**
American Red Cross- Learn About Blood Donation:  
www.redcross.org/services/biomed/blood/learn/
Aplastic Anemia & MDS International Foundation:  
www.aplastic.org
The National Marrow Donor Program:  
http://www.marrow.org/
John Hopkins:  
http://hopkins.med.jhu.edu/
Memorial Sloan Kettering Cancer Center:  
http://www.mskcc.org/

**Patient Pages:**
Alison’s Home on the Net:  
http://www.thegrid.net/asmaltz/
Rebecca’s PNH Pages:

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**Recommended Books**

**One Hundred Days, My Unexpected Journey from Doctor to Patient**

“I have PNH, and was stunned to find a book about my rare disease. This book does not go into great detail of living with PNH on a daily, weekly, monthly or yearly basis. Dr. Biro was fortunate enough to have a perfect match for his Bone Marrow Transplant. This book tells of his story through the BMT process, which I think is good for any one to read if they may have to have a BMT. It really explained a lot to me. Any one with PNH would benefit from reading his book. And it helps all of us with PNH to know there is a chance of a successful ending to PNH.” – Rebecca, living with PNH for 4 years.

**Tuesdays With Morrie**

“It gave so much inspiration to me. It has nothing to do with PNH but it gives the reader a lot of faith and courage.”
– Millie, living with PNH for 5 years.