2006 GLOBAL UPDATE
PROGRAM GUIDE

The Status of Research & Treatment
Annual Patient & Family Conference

Aplastic Anemia,
Myelodysplastic Syndromes (MDS) &
Paroxysmal nocturnal hemoglobinuria (PNH)

Hosted by the
Aplastic Anemia & MDS International Foundation, Inc.
August 2\textsuperscript{nd} – August 4\textsuperscript{th}
Nashville Airport Marriott, Nashville, Tennessee

800.747.2820 • WWW.AAMDS.ORG
This past May, 15-year MDS survivor and AA&MDSIF Chairman, Bob Carroll passed away. His passing has deeply saddened our Foundation, as well as the many patients with whom he shared his contagiously positive attitude, effervescent hope, and great love of life. Bob was a truly an inspirational man. An educator, humanitarian, husband, father, and ambassador for the AA&MDSIF, he will be remembered by all of us as a man of integrity, dear friend, and advocate to thousands of families touched by his incredible optimism and courage.

In 1991, at the age of 57, Bob was diagnosed with Myelodysplastic Syndromes and given only about two years to live. He often said that moment changed his life because it was in that minute that he committed himself wholeheartedly to make life better for other people. “I consider MDS to truly be a gift. It is my gift because it has taught me that relationships with others are what’s important in my life – nothing else.” It is this beautiful spirit and dedication to helping people that enabled Bob to beat his disease for so many years. While the details of his tireless acts of generosity and inspiration were unknown to many of us, the spirit that animated them is one we witnessed – and were moved by - every day.

Immediately after his diagnosis, Bob took the same step that many patients do – he searched for information about the disease. His research brought him to us, the AA&MDSIF, and immediately joined our team helping patients. For 14 years Bob worked on behalf of the AA&MDSIF; the past six as President, and, most recently, as Chairman of the Board. But it will be his unofficial role that we will miss the most – advocate, advisor, cheerleader, godfather, mentor, friend, and source of inspiration to staff and board members, patients and families, doctors and nurses, and to all of us fighting bone marrow disease. During his tenure, the AA&MDSIF expanded to offer services to patients around the world. His experience in Russia and Sri Lanka helped pave the way for the AA&MDSIF to become an international resource and assist patients and doctors with limited access to medical resources.

Bob left a world that is much improved because he lived in it. There are schools in Crimea and Sri Lanka that bear his name. There are patients alive because Bob found a way to get them medical supplies. There are hundreds of young people in his home state of Connecticut who are dedicating their lives to donating blood and platelets. And there are countless numbers of us who are stirred to fight the battle against bone marrow disease. This is Bob’s legacy, which will stretch far into the future.

To honor this legacy, Bob’s wife, Marie, and his sons, Robert, Jr. and Aric, have joined the AA&MDSIF board and staff to establish the Robert Carroll Legacy Fund to benefit the Aplastic Anemia & MDS International Foundation.

The Robert Carroll Legacy Fund is dedicated to Bob’s passion for helping patients and his devotion to our mission of leading the fight against bone marrow disease.

The Robert Carroll Legacy Fund will raise money to increase patient support, advocacy, and research.

The Robert Carroll Legacy Fund will help save lives, help families, and give hope to thousands around the world. Bob’s spirit will live on in the AA&MDSIF as well as in each of us that carry his love and hope forward. Please make a tax deductible contribution to The Robert Carroll Legacy Fund via AA&MDSIF.

Sincerely,

Mariantine Carroll
Wife of 36 years

Marilyn Baker, M.S.
President

P.S. View Bob’s final inspirational speech on our web video at www.aamds.org
DEAR FRIENDS,

Like many of you, when I was diagnosed with aplastic anemia, I had never heard of the disease before. As many of you did, I searched for information about the disease. My thirst for information connected me with the Aplastic Anemia & MDS International Foundation. AA&MDSIF immediately provided me with valuable medical information, assistance, and support...even though I lived across an ocean in Japan on an international assignment. Fate brought me back to live in the Washington, DC area where I eagerly joined their Board of Directors.

After the passing of Bob Carroll, I was honored to be asked to serve as Chairman of the Board. As Chairman I have a vision of continuing our proud tradition of being the leader in patient support, advocacy, and funding medical research to find a cure for bone marrow diseases. We will continue:

- Serving as the number one resource for medical information and support services,
- Expanding our quest to join patients, families, doctors, nurses, pharmaceutical companies, and government into a single team united in the mission to cure bone marrow disease,
- Operating with the absolute highest ethics and quality of service.

There is no doubt in my mind that the AA&MDSIF is strategically positioned.

As one who personally knows the importance and benefits of patient assistance and advocacy, I pledge to you my unwavering dedication and support to our common cause. I am very passionate about giving back to the organization that helped me and through my treatment and remission. Fellow Board members share this same passion.

I urge each of you to join our cause — there is so much to do! We can accomplish so much more by working together than we can alone. We have achieved major advances in the past couple of years, and we will do even more in the years to come.

Sincerely,

Neil Horikoshi, Esq.
Dear Friends:

I am delighted to welcome you to the 21st Annual Patient and Family Conference here in Nashville, Tennessee. Everyone at Aplastic Anemia and MDS International Foundation have been looking forward to seeing old friends and meeting new ones at this year's conference. Thank you for making such a big effort to be with us this year! In return we promise you an extremely informative, productive, and enjoyable couple of days together.

You will have the opportunity to hear from medical experts and personally ask them your questions. You will also have access to another important resource, the 90 nurses who will be joining us on Friday. And as always, there will be plenty of time to make friends with other patients and family members.

Sadly, our beloved Chairman and friend, Bob Carroll passed away this spring. For the past 14 years our Foundation was fortunate to have had Bob's leadership, hard work, integrity, inspiration, and love. Thousands of patients benefited from his incredible positive attitude and words of hope. Many of you today remember him at our past 14 Conferences...always smiling, always happy, always trying to help everyone he met. We continue to treasure Bob's love for life and his impassioned commitment to our cause. I can think of no better way to say thank you than to keep his hope, his energy, and his passion alive throughout the AA&MDSIF family.

So, keep Bob's positive outlook smiling over us the next couple of days. Gather all the latest medical findings—we have some of the finest medical minds with us. Absorb every bit of emotional support you can carry home with you—there are more than enough friends to go around. Build relationships that start today and will last a lifetime. Remember that we are a powerful group of people gathered here together —every member is precious, every minute counts.

Be Well,

Marilyn Baker, M.S.
President
CONFERENCE AGENDA

Important Note: frequent schedule changes may occur without warning
Registration Name Tags MUST be worn at all times for entry

WEDNESDAY, August 2

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<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>3:00pm</td>
<td>Registration Begins</td>
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<tr>
<td>5:00pm</td>
<td>Cocktail Party</td>
<td>Cumberland Ballroom</td>
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<tr>
<td>5:30pm</td>
<td>Exhibit Area Opens</td>
<td>Salon G</td>
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<tr>
<td>6:00pm</td>
<td>Dinner</td>
<td>Cumberland Ballroom</td>
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<tr>
<td>7:00pm</td>
<td>Welcome</td>
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<tr>
<td>7:30pm</td>
<td>Patient Advocacy Efforts – Mark Veith</td>
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<td></td>
<td><strong>Sharie Bardo and Drew Bourke, Nashville’s coolest</strong></td>
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<td></td>
<td><em>singer/songwriter/musicians, welcome us to Nashville and country music!</em></td>
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<tr>
<td>8:30pm</td>
<td>Mingle with patients &amp; families</td>
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THURSDAY, August 3

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<thead>
<tr>
<th>Time</th>
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<th>Location</th>
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<tbody>
<tr>
<td>7:30am</td>
<td>Breakfast &amp; Registration</td>
<td>Cumberland Ballroom</td>
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<tr>
<td>8:00am</td>
<td>Exhibit Area Opens</td>
<td>Salon G</td>
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<tr>
<td>9:00am</td>
<td>Disease 101</td>
<td>Cumberland Ballroom</td>
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<td></td>
<td>Dr. Paolo Anderlini</td>
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<td></td>
<td>Theresa Donohue, MS, PA-C</td>
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<tr>
<td></td>
<td><strong>BREAK</strong></td>
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<td>Health &amp; Wellness Issues</td>
<td>Cumberland Ballroom</td>
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<tr>
<td></td>
<td>Dr. Paolo Anderlini</td>
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<td>Theresa Donohue, MS, PA-C</td>
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<td>Barbara Tripp, RN, MSN, AOCN</td>
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<td>Drug Reimbursement Issues – Medicare Part B/D</td>
<td>Cumberland Ballroom</td>
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<td></td>
<td>Angela Mitchell</td>
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<td>12:15pm</td>
<td><strong>Box Lunches to take to Workshop Rooms</strong></td>
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<td>1:00-3:00pm</td>
<td><strong>Workshops</strong></td>
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<td>MDS Patients “Newly Diagnosed”</td>
<td>Cumberland Ballroom</td>
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<td>MDS Patients “Long-term Diagnosed”</td>
<td>Cumberland Ballroom</td>
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<tr>
<td></td>
<td>PNH Patients</td>
<td>Chattanooga</td>
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<td></td>
<td>Aplastic Anemia Patients</td>
<td>Salon F</td>
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<tr>
<td></td>
<td>Parents</td>
<td>Jackson</td>
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<td></td>
<td>Family</td>
<td>Knoxville</td>
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<td>Children/Teens</td>
<td>Memphis</td>
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<td>Spouses/Partners</td>
<td>Salon H</td>
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<td></td>
<td>Bereavement</td>
<td>Hermitage Room</td>
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<tr>
<td>4:30pm</td>
<td>Cocktail Party</td>
<td>Grand Ballroom Foyer</td>
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<tr>
<td>5:30pm</td>
<td><strong>Dinner with patients, families &amp; nurses</strong></td>
<td>Cumberland Ballroom</td>
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<td>Awards</td>
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<td>Our Hike – Robin Grapa &amp; Patty Laatsch</td>
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<td></td>
<td>Salute to Nurses!</td>
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<tr>
<td>7:30pm</td>
<td><strong>Entertainment</strong></td>
<td>Cumberland Ballroom</td>
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<td><strong>Dr. Humor! Laugh along with a doctor turned patient turned comic!</strong></td>
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<tr>
<td>8:30pm</td>
<td>Mingle with patients, families &amp; nurses</td>
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FRIDAY, August 4

Agenda may change dependent upon doctor's flight schedules.
Over 80 hematology & oncology nurses will join us for the day!

7:00am  Breakfast  Cumberland Ballroom
8:00am  Exhibit Area Opens  Salon G
8:00am  Relationship Between AA, MDS & PNH - Dr. Phillip Scheinberg

**Session I - AA**
- Dr. Phillip Scheinberg
- Theresa Donohue, MS, PA-C

**Break**

**Session II - PNH**
- Dr. Carlos DeCastro
- Dr. Anita Hill

**Session III - MDS**
- Dr. Elihu Estey
- Dr. Mikkael Sekeres

**Session IV – BMT & Pediatric**
- Dr. Paolo Anderlini
- Dr. Melissa Rhodes

**Lunch**  Cumberland Ballroom

Question & Answers Session

3:00pm  Adjourn  Grand Ballroom Foyer
5:00pm  Cocktail Party  Salon D & E
5:30pm  **Dinner**  Salon D & E
6:30pm  Victory Stories
7:00pm  Entertainment

*Craig Duncan’s Smoky Mountain Band and The Rocky Top Revue*

Music and dancing!

8:00pm  Mingle and say good-bye to your new friends

SATURDAY, August 5

12:15pm - 9:30pm: Nashville Sightseeing Tour  Meet in Hotel Lobby
Gray Line Tour bus departs at 12:30pm sharp for the Country
Music Hall of Fame, Dinner at The Loveless Cafe, and a Show at
The Grand Ole Opry. Pre-registration was required for this tour.
This excursion is the complete and total responsibility of Gray Line Nashville Tours.
AA&MDSIF is not responsible for the quality of service, food, entertainment, etc. of this tour.
Aplastic anemia (AA) and myelodysplasia (MDS), while different, share certain characteristics such as ineffective and inefficient blood cell production in the bone marrow, leading to low peripheral blood counts and requirement for red cell and platelet transfusions. Patients are also at risk for infection and bleeding problems. While growth factor (erythropoietin, filgrastim) and medication (thalidomide, antithymocyte globulin, azacitidine, lenalidomide, cyclosporine, corticosteroids, etc.) can ameliorate blood counts and reduce transfusion requirements, bone marrow or stem cell transplantation (BMT/SCT) is the only treatment with known curative potential. BMT/SCT is a procedure where marrow cells from another person are collected and are infused following the administration of medications (and sometimes radiation) aimed at killing the damaged and defective marrow cells or preventing the recipient’s immune system from rejecting the transplant (“conditioning regimen”). The donated cells can come from a related (i.e. a blood relative, usually a brother or sister) or unrelated donor. However, this treatment is associated with serious risks and complications, such as graft-versus-host disease (GVHD) and infection, particularly in older patients. Some patients die because of the procedure. Moreover, only about 25% of patients have a HLA-matched sibling donor. The last decade, however, has witnessed new and important developments in BMT/SCT for both AA and MDS, providing patients with more treatment options.

In AA, the emphasis has been in improving the outcome of older patients receiving a sibling donor transplant with the introduction of new, “reduced-intensity” fludarabine-based conditioning regimens aimed at maximizing the chance of engraftment (“take”) while minimizing side effects and toxicity. This is also allowing more and more patients with no sibling donor to successfully receive a transplant from a matched unrelated donor. In these cases, conventional total-body radiation (TBI) was frequently used to minimize the high risk of graft rejection (partly related to sensitization due to multiple blood transfusions), and this is no longer necessary. This has reduced side effects and late complications, such as growth retardation, infertility and premature menopause, major issues in younger patients.

In MDS, where most patients are in the 60-70 year age range, the need for a safer, better tolerated transplant approach was even more pressing, as patients used to do very poorly with conventional bone marrow transplants. Newer conditioning regimens employing drugs as fludarabine and intravenous busulfan, sometimes referred to as “minitransplants”, are now more commonly employed, with less toxicity and risk for the patient. Even for patients without a matched sibling donors, the outcome has improved thanks to a variety of factors, including better typing techniques for donors, better GVHD prevention and treatment as well as supportive care (antibiotics, antifungals, antivirals, etc.). Still, for patients receiving a transplant from a matched unrelated donor the procedure continues to carry significant risks.

In short, BMT/SCT for AA has preserved and now expanded its role in patients with a sibling donor, and even patients without a matched sibling donor (particularly if young) should now be considered, probably sooner rather than later. BMT/SCT in MDS has shifted from an intervention of last resort to a procedure to be considered in many patients (including older ones), particularly if they have an HLA-identical sibling.
A major cause of symptoms in patients with PNH, MDS, and aplastic anemia is the failure of the bone marrow to produce blood cells. In the past few years, both basic science research and clinical trial research have led to new and improved treatments. Eculizumab has shown high promise for treating PNH and will hopefully be approved by the FDA in the next year. Revlimid, Vidaza, and decitabine are now a part of the treatment options available for patients with MDS. Despite these new therapies, much work still needs to be done. The best hope for curative therapies for all patients will be in continued research and participation in clinical trials. Because some of these diseases such as PNH are uncommon, a great deal of cooperation amongst academic centers will have to occur. Funding for clinical trials is also necessary for which we are always grateful to organizations such as the AA/MDS Foundation. Finally we must always remember the patients who volunteer for clinical research trials as they are the true heroes of medical progress.
The incidence of myelodysplastic syndromes (MDS) is likely to increase as the U.S. population ages. Nonetheless, there are no satisfactory treatments. Furthermore, standard classification systems fail to explain much of the variability in either the natural history of these illnesses or their outcome after treatment. These problems reflect a lack of knowledge of the "biology" of MDS. New methods for clarifying the molecular pathogenesis of MDS have recently become available. This Program Project Grant (PPG) brings together investigators with clinical, molecular biological and statistical expertise to address the problems noted above. The primary goal of the PPG is to develop new therapies for both low and high risk forms of MDS. Project 1 will explore the role of new targeted therapies in the treatment of high-risk MDS and assess the relevance to survival and quality of life of the new category "minor response" recently promulgated by an NCI Working Group. Therapy for low risk MDS (Project 5) will be based on Dr. Molldrem’s hypothesis that peptides derived from normal tissue proteins can be autoantigens for cytotoxic T-lymphocytes (CTL) if peptide overexpression breaks immune tolerance. Preliminary data supporting this hypothesis and suggesting that vaccination with proteinase 3-derived epitopes can induce remissions are presented. Project 5 patients developing high-risk MDS despite the vaccination strategy will be treated on Project 1. Both Projects will use a common approach to evaluate quality-of-life and both employ therapies directed at targets that are quantifiable in the laboratory, thus motivating statistical evaluation of relationships between laboratory-based endpoints and clinical outcome. The PPG's second goal is gain further insight into the molecular pathogenesis of MDS. Although activating mutations in FLT3 (Project 2), RAS or PTPN11 (Core B), or HOX gene overexpression (Project 4) occur in 10-25% of MDS patients, large population-based studies that simultaneously monitor these mutations, or attempt to uncover new ones (Project 2), as the disease evolves are lacking. Interactions between Projects 2, 4, and Core B should, for example, permit evaluation of the hypothesis implicit in the Gilliland-Griffin model that while activating "type 1" mutations that confer a survival advantage (e.g. RAS, PTPN11, FLT3) will regularly be accompanied by "type 2" aberrations that block differentiation (e.g. HOX gene overexpression), two type 1 mutations will not occur in the same patient. The role played by epigenetic phenomena, e.g. hypermethylation, in MDS pathogenesis/progression will be examined in Project 3; up to 30% of MDS patients have hypermethylated genes, but this phenomenon has never been examined in the context of activating mutations or HOX overexpression. The PPG's third goal is to enhance the ability to provide accurate prognoses. To do this we will test the hypothesis that the findings from the laboratory-based projects (Projects 2-5) will provide information that will complement the information provided by the FAB, WHO, or IPSS systems. Preliminary data for example suggests that hypermethylation of a gene known as Ril confers a particularly poor prognosis.
Stem cells are the primitive cells in the bone marrow from which all other circulating blood cells are produced i.e. the red cells that carry oxygen around the body, the white cells that fight infection, and platelets that help the blood to clot. Paroxysmal nocturnal hemoglobinuria (PNH) is an acquired disorder in which a mutation affecting a gene called PIG-A occurs in a stem cell in the bone marrow. This mutation results in loss of production of an ‘anchor’ called the GPI (glycosylphosphatidylinositol) anchor. This anchor normally attaches numerous proteins to the surface of blood cells (red cells, white cells and platelets). Therefore, the PNH cells produced from the stem cell with the mutation are lacking many of these proteins. Although PNH results from a mutation it is not inherited and therefore cannot be passed on to a patient’s children.

Probably the most important protein missing is called CD59. CD59 protects our cells, particularly red cells and platelets, from attack by complement. Complement is a series of proteins designed to fight and destroy foreign organisms such as bacteria and viruses. As PNH red cells have lost the protective CD59 they are susceptible to attack by complement. The complement proteins attack the red cells resulting in their breakdown (hemolysis) and often causes anemia. The hemolysis also causes the hemoglobin from within the red cells to be released and may be visible in the urine of some patients with PNH, as dark urine (hemoglobinuria). Lack of CD59 on PNH platelets means these cells are also attacked by complement resulting in activation of the platelets and this is likely to be the principle mechanism behind the thrombosis that occurs in PNH. Complement is more activated at the time of infections or stress meaning patients will experience worse symptoms (paroxysms) during these periods. However, there is a continual low-level activation of complement which will affect the red cells and platelets.

Treatments for PNH have traditionally relied on blood transfusions, folic acid supplementation, anticoagulation and some have occasionally used steroids. The most exciting event in PNH in recent years is the development of a targeted therapy for PNH, the drug eculizumab. Eculizumab blocks the complement proteins, and therefore the red cells in PNH should be protected from hemolysis. This should mean patients receiving eculizumab will find their urine clears and require less, or even no, red cell transfusions.

A pilot study using this drug in 11 patients with PNH was commenced in 2002 in Leeds, U.K. The results were dramatic. Patients found that their symptoms, and therefore quality of life, were much improved. Also, there was a significant fall in the number of transfusions that patients required with many not requiring a further transfusion (transfusion independence). The urine also cleared in these patients. Due to these promising results a Phase III worldwide trial (TRIUMPH) was completed in Europe, USA and Australia, in which 87 patients were randomized to receive either eculizumab or a placebo agent for 6 months. The results have now been reported and remain very positive. The marker of hemolysis, LDH, was significantly lower in the patients receiving eculizumab. These patients also had stable hemoglobin levels and decreased number of transfusions compared with patients not receiving the drug. Quality of life was also significantly improved. Patients have reported feeling ‘normal’ again and some have been able to return to full time work from previous unemployment due to ill-health. All patients who were on placebo for the trial went onto eculizumab after 6 months. The drug also appears safe and very well tolerated by patients.

Although not yet proven, there is a theoretical possibility that eculizumab should prevent, or at least reduce, the incidence of thrombosis in PNH. As the drug blocks complement proteins, the platelets will not become activated by this route.

Eculizumab is currently not licensed and is awaiting review by the Food and Drug Administration (FDA) and the European Agency for Evaluation of Medicinal Products (EMEA). However, the future for PNH looks very promising with the advent of eculizumab.
The Medicare Prescription Drug Plan also known as Medicare Part D, which began on January 1, 2006, was successful in getting 8% percent of the 43 million eligible Medicare beneficiaries to enroll in a Prescription Drug Plan (PDP). Now that Medicare beneficiaries have selected a PDP, millions of beneficiaries are facing new obstacles to obtaining access to drugs. Nearly 3.4 million beneficiaries are approaching the donut-hole, the gap coverage under which beneficiaries must pay 100% of their annual medication costs between $2,250 and $5,100. Patients that are reaching the donut-hole suffer from multiple chronic conditions and cannot afford the out of pocket expense to get them out of the donut-hole. Patients who are diagnosed with aplastic anemia and myelodysplastic syndrome (MDS) may find themselves facing the same obstacles to obtaining drugs because of the donut-hole.

In addition, patients enrolled in Medicare Advantage (MA) plans are experiencing the “bait” and “switch”. MA plans are covering drugs under the pharmacy benefit that are typically covered under Medicare Part B. Most MA plans offer limited pharmacy coverage, which means that if expensive drugs are included as part of the pharmacy benefit a patient can “max out” their annual prescription drug benefit. It is important for patients to understand the five major categories of Medicare Part B drug spending to recognize when a MA plan has made a policy decision to include Medicare Part B drugs under its prescription benefit. The five major categories of Medicare Part B drugs include: 1) drugs billed by a physician and typically provided in physician offices such as chemotherapy, 2) drugs billed by pharmacy suppliers and administered through Durable Medical Equipment (DME), such as respiratory drugs billed through a nebulizer, 3) drugs billed by pharmacy suppliers and self administered by patients such as immunosuppressive drugs and some oral anti-cancer drugs, 4) separately billable drugs provided through a hospital outpatient department, and 5) separately billable end stage renal disease drugs (ESRD). Despite the general limited coverage under Part B, the law specially authorizes coverage for many drugs to include oral anti-cancer drugs and oral anti-emetic drugs.

Patients should continue to be aware of “brown bagging” which is a term used to describe when an insurance company finds an inexpensive wholesale supplier of oncology drugs and has the supplier ship the drugs directly to the patient. This type of practice is very beneficial to the insurer but does not take into the consideration the impact to the patient which could include improper handling and storage of the product, and a delay in treatment because the product may not arrive to the patient’s home in time for scheduled treatment appointments.
Aplastic anemia and myelodysplasia (MDS) are both uncommon in children and often have different causes in children than in adults. While aplastic anemia and MDS in adults are most often idiopathic or related to medications, infections, or radiation exposure, almost half of children with aplastic anemia and MDS have underlying genetic disorders. It is important at the time of diagnosis to consider an underlying genetic disease, as this may affect the choice of treatment, expected outcome, and anticipated complications of treatment. Genetic diseases that must be considered include Fanconi anemia, dykeratosis congenita, Schwachman-Diamond syndrome, reticular dysgenesis, and amegakaryocytic thrombocytopenia. While these genetic disorders have typical physical manifestations, recent data shows that about half of patients with the gene defects of these disorders do not have any physical characteristics typical of the disorders. Furthermore, relatives of patients diagnosed with these genetic disorders are being found to have the same genetic defects but more mild symptoms. Because bone marrow transplant is often used to cure the patient, potential related donors must be screened if the patient has one of these genetic disorders.

MDS in children is generally considered a pre-leukemic condition; therefore, children with MDS are usually treated with bone marrow transplant in attempt to cure the disease before it progresses to leukemia. While a bone marrow transplant from a sibling or other related donor is best, advances in immunosuppressive medications and supportive care are making the outcomes of transplants from unrelated donors and cord blood almost comparable to related donor transplants. However, there are still some patients for whom bone marrow transplant is not feasible. For these patients, new therapies offer hope for long-term treatment.

Aplastic anemia is more common in children and young adults than in older adults. Children generally have better outcomes than adults. Bone marrow transplant results in cure in over 80% of children and adolescents with aplastic anemia. Immunosuppressive treatments that use a combination of anti-thymocyte globulin (ATG), cyclosporine, GM-CSF or G-CSF and steroids result in about 60-70% disease-free survival. If children relapse after response to these agents, re-treatment often results in a second remission. New treatments including novel immunosuppressive agents, targeted anti-cytokine treatments, and photopheresis are helping children who have relapsed and are now used in upfront treatment studies. These novel agents have the benefit of targeted therapy, causing less overall immune suppression and therefore lower risk of infection.

PNH in children is extremely rare, however, PNH must always be considered with a child who has aplastic anemia.

Children who have been successfully treated for aplastic anemia and MDS need follow-up care by a pediatric hematologist, and often by other subspecialists. The underlying disease, as well as medications used in treatment, place patients at an increased risk of developing cancers, secondary blood diseases such as PNH, growth impairment, delayed puberty, iron overload, damage to the heart, liver, and kidneys, as well as psycho-social issues particular to children with life-threatening diseases. These secondary problems are scary, but they are treatable if diagnosed early. Close follow-up at a pediatric center may allow survivors to live a more normal life.
CURRENT PRACTICE IN APLASTIC ANEMIA

Acquired aplastic anemia (AA) is characterized by pancytopenia with a hypocellular, often "empty", bone marrow. Hematopoiesis is severely reduced in all AA, as observed in bone marrow specimens, CD34 cell counts, magnetic resonance imaging, or in functional studies of progenitors. Clinical and laboratory studies suggest that most acquired AA is secondary to immunologically mediated destruction of hematopoietic cells by cytotoxic lymphocytes (CTL) and their cytokine products, especially interferon-γ (IFN-γ) and tumor necrosis factor-α (TNF-α).

Patient’s complaints derive from their low blood counts. Bleeding is an alarming symptom, even if usually minor in character: gum oozing, nosebleeds, easy bruising, heavy or irregular menses often precipitate the first visit to a physician. Frequent are the nonspecific symptoms of chronic anemia: fatigue or lassitude, shortness of breath, and ringing in the ears; older patients may have chest pain or congestive heart failure. Surprisingly, serious infection is unusual at presentation. Notably absent also are systemic symptoms of weight loss, failed appetite or fever, and most patients feel well despite their very low blood counts.

Clinically, aplastic anemia may coexist or appear to evolve to other hematologic diseases which are characterized by proliferation of distinctive cell clones, as in paroxysmal nocturnal hemoglobinuria (PNH) or myelodysplasia (MDS). Dark urine from the presence of hemoglobin may accompany PNH. The presence of tiny clones at the time of diagnosis of aplastic anemia, detected using extremely sensitive assays--phenotypic (flow cytometry for PNH) or cytogenetic (fluorescent in situ hybridization for MDS)--also creates problem of disease classification and patient diagnosis. Most PNH clones are small and do not lead to clinical manifestations of hemolysis or thrombosis but classic PNH can be dominated by marrow failure (the “aplastic anemia/PNH syndrome”) and all PNH patients show evidence of underlying hematopoietic deficiency. Stereotypical patterns of aneuploidy develop in a minority of patients over time: monosomy 7 or trisomy 8 are most characteristic of MDS.

The introduction of anti-thymocyte globulin (ATG) treatment in severe AA in the late 70's has played a significant role along with hematopoietic stem cell transplantation (HSCT) in the treatment of this disease. Since HSCT is often not a viable option due to age or lack of an HLA-identical sibling, immunosuppressive therapy is often used as initial treatment strategy. ATG and cyclosporine is standard therapy. About 2/3 of patients improve to transfusion-independence and, overall survival rates at 5 years are comparable to HSCT. Even after hematologic response to ATG, blood counts may fall, especially on withdrawal of cyclosporine. Reinstitution of cyclosporine usually suffices, but retreatment with ATG may be necessary. Evolution to a clonal hematologic disease occurs in about 10-15% of patients over the decade after initial therapy. Immunosuppression is almost always preferred in older patients, especially if the neutrophil count is not severely decreased. Our efforts at NHLBI have been in optimizing the immunosuppressive drug regimen in order to further improve the response rate. In the last 5 years, we've tested the addition of mycophenolate mofetil (MMF) and sirolimus (Rapamune) to standard horse ATG in untreated patients. In patients refractory to initial horse ATG, the use of rabbit ATG and Campath is currently being evaluated.
The Myelodysplastic Syndromes (MDS) are a group of bone marrow disorders that often result in inadequate blood counts. Within the bone marrow, an excessive number of abnormal (dysplastic) cells grow and, either directly or through the production of chemicals (cytokines), cause normal bone marrow cells to die prematurely (a process called apoptosis). A new diagnosis of MDS is made in between 12,000 and 20,000 people in the United States each year, making it perhaps the most common disorder of the bone marrow.

In broad strokes, MDS can be divided into two major categories: Low-grade or early MDS, in which the premature death of normal bone marrow cells predominates; and high-grade, or Advanced MDS, in which the excessive growth of immature cells and the premature death of normal cells occur.

Therapies for MDS, then, have different mechanisms of actions and different treatment goals, depending on the MDS subtype. For low-grade MDS, therapies are directed towards stimulating the growth of normal cells (through the use of growth factors), and towards stopping the effects of cytokines (with agents such as lenalidomide or thalidomide). Treatment goals include minimizing transfusions of blood or platelets and maximizing quality of life, and therapies are typically started when a person starts to require blood transfusions, or will shortly.

For high-grade MDS, therapies stop the growth of dysplastic cells (with hypomethylating agents such as 5-Azacytidine or decitabine), or even eliminate all bone marrow cells and replace them with a transplanted normal bone marrow (as would occur with a bone marrow or stem cell transplant). Therapeutic goals center on bringing about a remission, possibly improving overall survival, and again, maximizing quality of life.

Most therapies for MDS have side effects that include a transient lowering of blood counts, and these side effects have to be balanced with the potential efficacy of these drugs, and the balance between side effects and quality of life. Supportive therapies, such as blood transfusions, antibiotics, and iron lowering agents or even growth factors, may be used along with other therapies for MDS. Clinical trials with newer drugs are often available, and may be as viable an option as more established therapies.
In 2005, the Aplastic Anemia & MDS International Foundation launched a new series of initiatives designed to raise greater awareness of bone marrow diseases, reach new patients, and increase federal funding into new treatments and cures. The cornerstone of these initiatives to date has been the **Bone Marrow Disease Resolution (H.Con.Res. 179)** introduced in the U.S. House of Representatives on June 16, 2005. This bipartisan resolution was introduced by Representatives Sherrod Brown (D-OH) and Michael Bilirakis (R-FL). The resolution calls on the federal government to take a more active role in the fight for a cure for bone marrow diseases. The resolution, which is cosponsored by 45 Republican and Democratic Members of the House, specifically cites the role of the Foundation in this endeavor, stating that “families coping with bone marrow failure diseases should be linked to support networks and counseling and information services provided by non-profit organizations like the Aplastic Anemia & MDS International Foundation.”

AA&MDSIF has also been working to recruit new champions on Capitol Hill to help raise several important research issues with the National Institutes of Health (NIH). Working with the Senate Appropriations Committee, AA&MDSIF secured directive language in the fiscal year 2006 Labor-Health and Human Services Appropriations bill for several NIH institutes. This year, AAMDSIF worked with the House Appropriations Committee to **secure language calling on the NIH to initiate a study in Asia** into the disproportionate prevalence of bone marrow diseases on the Asian continent.

In addition to strengthening its ties to the NIH, the Foundation has been working to establish new partnerships with other federal agencies, including the Administration on Aging, the Centers for Disease Control and Prevention (CDC), and the Veterans Administration. These partnerships have been forged to reach bone marrow disease patients who may not have had contact with AA&MDSIF:

Working with like-minded organizations, including other rare disease groups, the AAMDSIF has also participated in coalitions that have been formed in Washington, D.C. to fight for higher overall funding levels for the NIH and CDC. If proposed cuts to these agencies come to fruition, it will be difficult for organizations like the Foundation to achieve their specific funding objectives within these respective agencies.

AA&MDSIF’s government relations initiatives have succeeded to date thanks to a focused advocacy strategy and the grassroots involvement of bone marrow disease families. As these initiatives progress through an increasingly difficult political and fiscal environment, it becomes more even more critical for the entire bone marrow disease community to get involved at a grassroots level. Focused, persistent grassroots advocacy is the key to raising greater awareness in Congress and the general public, and increasing federal funding for new treatments and cures.
Join Us for Nightly Entertainment & Fun!

Shari Bardo and Drew Bourke welcome us to Nashville, Tennessee Wednesday evening...

The Music City Songwriter Experience

Join two of Nashville’s coolest singer/songwriter/musicians as they give you an insider’s view of how a song is written. Sharie Bardo and Drew Bourke demonstrate all the elements that go into building a song from scratch in this interactive experience as participants contribute ideas, melodies and lyrics. With professional guidance, guests literally write a song – from intro to finale - on the spot. Are you listening, Garth or Reba?

Don’t miss “Dr. Humor” Thursday Night ...

Dr. Stuart Robertshaw, Professor Emeritus of Psychology and Education at the University of Wisconsin-La Crosse and an Attorney, will share with you his personal journey which began in September, 1987 when he started a review of the research on the benefits of humor. On June 28, 1990, he announced the formation of the National Association for the Humor Impaired. The Association has received national attention from the press in over 144 newspapers, 180 radio stations and has been featured in magazines such as Family Circle and Mature Outlook. Dr. Robertshaw, whom the press refers to as “Dr. Humor,” currently serves as President and Chief Executive Officer of the Association. “Dr. Humor” will share with you what he has learned about the psychological and physiological benefits of humor and laughter as they relate to taking care of ourselves.

Craig Duncan’s Smoky Mountain Band and Rocky Top Review for a “Taste of Nashville” on your last night in Tennessee!

Craig Duncan’s Smoky Mountain Band performs traditional Tennessee music at its best. The 4-member band features multi-instrumentalists playing fiddle, mandolin, banjo, guitar, bass and drums in a unique blend of folk, bluegrass and country stylings. Craig Duncan has been the featured instrumentalist on over sixty record albums with sales in excess of five million. He has produced numerous recordings in a variety of musical styles. He is a member of the North American Fiddler’s Hall of Fame and is recognized internationally for his many books and arrangements published by Mel Bay Publications.

The Rocky Top Revue, championship cloggers, perform an All-American Riverdance. With an exhibition of incredible footwork and colorful style, they can bring up audience members for an interactive treat or lead the entire audience in a “cloggin’ conga line”. RTR always raise the roof and bring the house down.

Nashville Sightseeing Tour on Saturday, August 5th

AA&MDSIF has arranged for a Nashville Sightseeing Tour. Advance registration was required. This special tour includes bus fare from the hotel, admission to the Country Music Hall of Fame, Dinner at the Loveless Cafe, and a Grand Ole’ Opry show. The group should meet in the hotel lobby at 12noon on Saturday to be sure not to miss the buses.
OUR EXPERTS EMPOWER YOU WITH ANSWERS, SUPPORT, and HOPE…

Marilyn Baker, M.S.  
President  ✉ baker@aamds.org
Marilyn joined the Aplastic Anemia & MDS International Foundation (AA&MDSIF) in 1990. She has worked with thousands of patients and their families as they manage their illnesses, make treatment decisions, and cope with the emotional issues of living with a rare disease. She has also raised millions of dollars for patient support and medical research. Under her leadership, the AA&MDSIF has grown and evolved to become the world’s largest patient advocacy and support organization focused on bone marrow diseases. Today, as the leader of a worldwide movement to combat bone marrow disease, Marilyn focuses her energies on building awareness and understanding of bone marrow diseases, establishing strong international, corporate, and government partnerships, and working with academic and community based hematologists and nurses to better patient care.

Jennifer Krammes  
Director  ✉ krammes@aamds.org
Jennifer manages the products and services of the AA&MDSIF, ensuring that patients, families, health practitioners, and other partners receive immediate, direct, and highly accurate information and assistance when they contact us. She also coordinates public outreach and awareness activities and assists the entire network of patients, families, nurses, doctors and other partners to mobilize attention and resources for bone marrow disease. Jennifer has vast expertise in the areas of non-profit management and event coordination during her 12 years as executive director of several non-profit organizations. Since she joined the AA&MDSIF in 2004, Jennifer has increased our capacity to serve the growing number of patients, families, and health care providers who turn to us for information and support.

Cecelia Petro  
Patient Information Specialist ✉ petro@aamds.org
Cel is a dedicated partner in our patients’ efforts to maintain their health. Each patient or family member who reaches Cel on the phone receives extensive information on bone marrow diseases, treatment options, and research findings—and with these resources the comfort of a compassionate and active listener. A knowledgeable and experienced research librarian, Cel continuously monitors research on bone marrow disease. She performs literature searches for patients on more complex questions and issues. She also keeps up-to-date on other issues that affect bone marrow patients such as Medicare and prescription reimbursements. Recognizing that nurses are among the most powerful and accessible allies in the fight against bone marrow disease, Cel works with nurses round the country to provide them with the information and support to improve care for bone marrow patients.

Katherine Baer  
Patient Information Specialist ✉ baer@aamds.org
Katherine is the newest member of the AA&MDSIF team. She brings with her many years of in-depth experience as a special librarian. She uses her varied research skills to present patients with the knowledge they require: providing patients and family members with personalized assistance, information, and resources on the diseases. She performs literature searches for patients on questions and issues they face on a day-to-day basis while keeping up-to-date on all issues that affect bone marrow patients.

Elizabeth Bradley  
Clinical Trials Educator ✉ bradley@aamds.org
Beth provides thousands of patients who call the AA&MDSIF with access to clinical trials and other patients. Beth monitors clinical trials that are being conducted around the country and helps link patients to these studies when appropriate. She also helps guide patients to medical experts when are in need of a second opinion. She manages global network volunteers, linking together patients and families around the world to share hope and information. For over 10 years, Beth has worked as a patient advocate in a variety of health care setting. She brings a thorough understanding of patients’ medical and non-medical needs to her work of linking patients with knowledgeable volunteers and local support groups. In her own words, Beth is part of a formidable team that gives our patients information, hope, and the resources to take charge of the disease—and focus on their quality of life.
Kathy Fisher
Financial Administrator ● fisher@aamds.org
If an organization is very lucky, it takes someone like Kathy who works behind the scenes to make sure everything runs smoothly. Kathy works with patients, family members, and friends to set up memorial gifts and other donations that support our research and patients assistance work. Kathy works with patients and family members who are interested in attending our annual conference. Kathy also manages our travel fund, which enables patients to participate in distant clinical trials. With an extensive background in office management and accounting, Kathy is more than capable of dealing with the daily emergencies that arise in a place that helps people to cope with complex and rare diseases. With quiet grace and good humor, Kathy works with patients, health practitioners, and colleagues to make sure that the important details—the timely thank you, the word of encouragement, and the gentle tribute—are handled with care.

Yvonne Finne
Project Coordinator ● finne@aamds.org
Yvonne manages the many projects that are going on at any given time—from producing a new brochure to making travel arrangements for the doctors who will be speaking at our annual patient conference. A skilled and experienced project manager with years of experience, Yvonne makes sure that schedules are met, that resources are used wisely, and that our activities are completed with care, attention to detail and with the excellence our patients have come to expect. AA&MDSIF relies on partners to fight with us against the many challenges of bone marrow disease. Yvonne makes our joint projects work—from the drawing board to final delivery.

Anita Henry
Administrative Assistant ● henry@aamds.org
Anita provides patients and family members with packets of information during those first days—when they have just received a diagnosis and want to know more. Anita also works with health practitioners to ensure that clinics and medical offices are supplied with information for their patients. Every week, Anita sends out hundreds of copies of our materials targeted to the specific needs of those who contact us. Our materials cover the spectrum of disease-related information from basic explanations of the diseases and their symptoms to guides on managing treatment options. Anita draws on over 20 years of customer service management to make sure every patient, family member, friend or health care professional receives accurate and timely information with the care and attention that are the hallmarks of our work.

Annie Wiederanders
Intern ● associate@aamds.org
Annie is a 21-year-old aplastic anemia patient, who is working with AA&MDSIF as an intern through December. She is a senior at the University of California, Santa Cruz, majoring in Community Studies with a minor in Legal Studies. When first diagnosed, Annie benefited from AA&MDSIF services and now wants to give back by helping the foundation and other patients. Annie will focus on government advocacy, and will support networking communication through the website and other projects. She would also be happy to speak with young adult patients to offer an amount of help she can.
Special thanks from AA&MDSIF to the following sponsors who have made this year’s Patient & Family Conference possible

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Bone Marrow Failure Disease Consortium

To address the challenges in diagnosing and treating bone marrow diseases, the National Institutes of Health (NIH) has awarded a $4.5 million grant to a group of expert physicians and their teams called the Bone Marrow Failure Disease Consortium (BMFDC). These distinguished and recognized authorities in treating bone marrow diseases are holding clinical studies at four centers of excellence across the US to find advances in treatment options for patients. The BMFDC functions under the umbrella of the NIH's Rare Diseases Clinical Research Network (RDCRN) along with nine other consortiums which are part of this $71 million grant to study numerous other rare diseases.

"Funding research on rare diseases is a vital aspect of the NIH mission," said NIH Director Elias A. Zerhouni, M.D. "By encouraging cooperative partnerships among the investigators at these centers we hope to accelerate the development of diagnostics and treatments that will benefit these important patients."

The BMFDC studies aplastic anemia, myelodysplastic syndromes, paroxysmal nocturnal hemoglobinuria, and single lineage cytopenias such as large granular lymphocyte leukemia and pure red cell aplasia. While these and other rare diseases affect an estimated 25 million people in the U.S., the patient populations are widely distributed geographically. Research therefore requires significant collaboration among scientists and research centers, which is one of the primary ideas behind the RDCRN and BMFDC.

"The network [RDCRN] will facilitate increased collaboration and data sharing between investigators and patient support groups working to improve the lives of those affected by these diseases and potentially prevent or eliminate these diseases in the future," said Stephen Groft, Pharm.D., director of NIH's Office of Rare Diseases.

Another important goal of the BMFDC is to enhance the availability, accessibility, and quality of information offered to patients and health care professionals. To coordinate efforts in this area, AA&MDSIF serves as a clearinghouse for patient education about clinical research studies and for information about current advances in research. For this role, the AA&MDSIF draws upon its wide reach and extensive information resources as a patient advocacy group focused on bone marrow diseases.

BMFDC Patient Contact Registry

The BMFDC created a Patient Contact Registry where patients and their families can sign up to be contacted in the future about clinical research opportunities. Every patient who is considering participating in a clinical study may want to explore this opportunity. To learn more about the BMFDC Patient Contact Registry, visit [http://rarediseasesnetwork.epi.usf.edu/bmfdc/index.htm](http://rarediseasesnetwork.epi.usf.edu/bmfdc/index.htm) where you can complete an online registration form, or call (866) 313-9879 where you can join by telephone.

BMFDC Clinical Trials

There are several BMFDC studies now recruiting patients, including a longitudinal study of bone marrow failure syndromes and cytopenias. Other protocols include a phase I study of lenalidomide in combination with azacitadine for patients with advanced MDS, a phase I/II study of sirolimus and cyclosporine in patients with refractory aplastic anemia, and a phase II study of tipifarnib in patients with Large Granular Lymphocyte (LGL) Leukemia. For more information, visit [http://rarediseasesnetwork.epi.usf.edu/bmfdc/index.htm](http://rarediseasesnetwork.epi.usf.edu/bmfdc/index.htm) or call Robin Heggeland, RN of the Cleveland Clinic Foundation at (216) 445-7648.

Personal Assistance in Understanding Clinical Trials

The AA&MDSIF employs a full-time Clinical Trials Educator, Beth Bradley, who is devoted to helping you learn about the purpose of clinical trials, what to expect if you choose to participate in a study, and the risks and benefits to consider. Beth also maintains the AA&MDSIF Clinical Trials Listing, which contains descriptions of current research studies, names and locations of hospitals, and phone numbers for expert physicians who conduct these studies. Contact Beth at (800) 747-2820 or bradley@aamds.org.
GLOBAL VOLUNTEER NETWORK FORM

DATE: ___________________  PATIENT'S CURRENT AGE: ________________

TYPE OF DIAGNOSIS (Check One):  [ ] Aplastic Anemia  [ ] PNH
[ ] Myelodysplastic Syndromes  (Type): ______________________

NAME OF PERSON FILLING OUT FORM: ____________________
RELATIONSHIP TO PATIENT: ____________________ (self, spouse, parent, brother, daughter, etc.)

ADDRESS: __________________________________________

CITY: ___________________ STATE: ___________________ ZIP/POSTAL CODE: ______________

COUNTRY: ___________________

TELEPHONE: ( ______ ) _______ EMAIL ADDRESS: ______________________

BEST TIME TO REACH YOU: ______________________

PATIENT’S NAME: ______________________________

AGE & YEAR WHEN DIAGNOSED: AGE: ___________ YEAR: ___________

PATIENT’S CURRENT HEALTH STATUS: ____________________________

YEAR & HOSPITAL, CITY & STATE OF TREATMENT: __________________________

TYPE OF TREATMENTS: ____________________________

IF BMT, WHAT TYPE OF DONOR? (Check One):  [ ] RELATED  [ ] UNRELATED  [ ] STEM CELL

PERSONAL EXPERIENCES THAT MAY BE HELPFUL TO SHARE WITH OTHER PATIENTS AND FAMILIES:

__________________________________________________________________________
__________________________________________________________________________

LANGUAGE SKILLS (NON-ENGLISH):

__________________________________________________________________________

I HEREBY GIVE permission to the Aplastic Anemia & MDS International Foundation, Inc., (AA&MDSIF) to distribute any of the information that I have provided on this questionnaire to any individual or organization wanting to contact me regarding my treatment experiences. I understand that the information is not confidential and AA&MDSIF is not responsible for any of the interactions I may have with individuals or organizations that have used the information to contact me. I also acknowledge that the AA&MDSIF are not responsible for anything that such an individual or organization may subsequently do with the information provided. I HEREBY RELEASE the AA&MDSIF from any liability associated with the use and distribution of such information.

CONSENTED TO THIS: ______________________
SIGNATURE

DATE ______________________
Workshops Explained …

Finding another patient in your town may be as rare as your disease. Our conference workshops serve an important purpose by offering patients, parents, family and other caregivers the unique opportunity to connect with a group of individuals walking in their shoes. Guided by licensed counselors, these workshops will let you exchange information, share your feelings, discuss special issues, give and receive encouragement, and develop a network of new friends to support you long after the conference ends!

Patients:
- Aplastic Anemia Patient Workshop
- PNH Patient Workshop
- MDS Patient Workshop A (for patients diagnosed LESS THAN 3 YEARS)
- MDS Patient Workshop B (for patients diagnosed MORE THAN 3 YEARS)

Patients who have each disease gather together in their own meeting area to share their experiences and discuss issues related to the physical, emotional, and lifestyle challenges of living with their disease, and learn about successful coping strategies.

Patients in the two MDS Workshops will join together during the second half of the workshop, which will be guided by two facilitators.

Parents Workshop
Parents will meet to discuss various approaches for dealing with the needs and responsibilities of providing daily emotional and physical support to their child, including ideas for keeping a healthy outlook while balancing the needs of the child with the requirements of everyday family life.

Children & Teens Workshop (for patients under 18)
Young people who have a bone marrow disease talk about what it’s like living with their disease with others who have “been there”. This Workshop gives children and teens an opportunity to make friends and encourage each other.

Spouses/Partners Workshop
Caregivers who support their spouse or partner in living with bone marrow diseases discuss common needs and concerns with people who truly understand this difficult situation, including ways to maintain positive communication and healthy relationships while caring for yourself, your partner, and your family.

Family Workshop
Other caregivers and loved ones who support an ill family member will meet to share experiences, talk about methods of coping, and consider ways that family and friends can help by maintaining positive communication and interaction.

Bereavement Workshop
Those who are grieving the loss of a loved one from a bone marrow disease will be guided by an experienced bereavement counselor to share experiences, reflect, and give and receive support.
Workshops Facilitators …

**Aplastic Anemia Patient Workshop**  
*Mary Winslow, LCSW*  
Mary is a licensed clinical social worker who has many years of experience counseling and guiding support groups. She is the Patient Services Manager for the Nashville chapter of the Leukemia & Lymphoma Society where she leads their cancer support groups.

**PNH Patient Workshop**  
*Ken Lass, PhD*  
Ken is a psychologist who has been in practice serving families and individuals for nearly 20 years. He has conducted numerous wellness groups and parenting classes, and he is also involved in outreach work.

**MDS Patient Workshop A (patients diagnosed LESS THAN 3 YEARS)**  
*Deborah Wolkhamer, LCSW*  
Deborah has years of experience counseling, leading support groups, and helping families as a Social Worker. She currently works at Vanderbilt Children’s Hospital in the Social Work Department.

**MDS Patient Workshop B (patients diagnosed MORE THAN 3 YEARS)**  
*Will Hutchins, Licensed Professional Counselor*  
Will has earned a Master Level Divinity Degree and has been in the mental health field for 35 years. He has extensive experience conducting support groups.

**Parents Workshop**  
*Mark Wiederanders, PhD*  
Mark is a research psychologist who conducts research in the mental health system and has counseling experience as well. We are particularly lucky to have Mark facilitate our Parents Workshop this year because he has firsthand experience with bone marrow disease – Mark’s daughter has aplastic anemia.

**Children & Teens Workshop (for patients under 18)**  
*Shalene Grinder, LCSW*  
Shalene has extensive experience counseling and leading support groups for children and teens, as well as parents. Along with her years of traditional office-based counseling experience, Shalene has also worked at Vanderbilt Hospital in the mental health field.

**Spouses/Partners Workshop**  
*Caron Petersen, MSW*  
Caron has worked for 30 years in social services and she currently runs a statewide training project in Nashville. She has conducted countless seminars, most of which focus on wellness and possibilities, and she is well known for her energy and humor when facilitating groups.

**Family Workshop**  
*Vicki Williams, MSW, ACSW*  
Vicki has worked in the mental health and social service field for years and she has extensive experience leading workshops. Vicki is currently a professor and the Program Director of Social Work at Tennessee State University.

**Bereavement Workshop**  
*Holly Montgomery, LCSW*  
Holly has many years experience counseling individuals, couples, children, and families. She has worked with national hospitals and she leads a cancer support group for Gilda’s Club.
Salute to Nurses! …

“Salute to Nurses!” will be a very special presentation celebrating the wonderful nurses in attendance and the nursing profession. This is your chance to salute your nurse; tell your short, abbreviated version, of how your nurse made the difference in your treatment! Nurses will be joining the Patient & Family Conference – let’s really let them know how incredible they really are to all of us. It is a truly special part of the evening for everyone to be a part of ~ be sure not to miss it!

Following is a fun excerpt from an 1895 Look at Nursing:

“The Nurse’s Qualifications ~ The qualifications required to be a successful nurse are necessarily of a high order and this applies not only to the trained nurse, but to her embryo sister who wishes to adopt nursing as a calling.”

“…The best type of nursing girl is one who is tall and strong, and who has a certain suppleness of movement. One, who is accustomed to play lawn-tennis, who can ride, and skate, and row, makes the best material. If she can dance, especially if she is an enthusiastic dancer, it is a great advantage, for graceful carriage is a thing to be cultivated, and nothing is more distasteful in a sick-room than a suspicion of clumsiness. …”

“A nurse who aspires to rise in her profession should have a soft and evenly modulated voice, for harsh notes jar on the ears of sensitive patients. With regard to her general education she must be able to speak her own language correctly, and if she has a smattering of French and German so much the better. She should be able to write a good hand, and should have an elementary knowledge of how to keep accounts. Respecting her moral attributes, it may be said that a girl who has been brought up in a country parsonage, and has had little experience of the world, is hardly fitted for hospital work. …”~ An 1895 Look at Nursing [http://ENW.org/1895_Nursing.htm] is a web article presented by: Emergency Nursing World! [http://ENW.org] ©Tom Trimble, RN [Tom@ENW.org]

Visit the Exhibit Area …

Find out the latest news first hand from drug companies you want to hear from! Pharmion, Novartis, Celgene, Alexion, MGI Pharma will all be there to answer your specific questions at your convenience. Get the information you have wanted to find out.

In addition, the Exhibit Hall has wellness information that will benefit you in so many ways; AA&MDSIF Information and Support, Borders Books, Caring Bridge, Lotsa Helping Hands, and Healing through Expression. Learn more about these organizations and how they can enhance the quality of your life.

This area of the Conference will open on Wednesday at 5:30pm and remain open through dinner. It will be open at 8:00 am on Thursday and Friday and remain open throughout dinner in order for you to find the time to visit and collect the information you are looking for.
Our Hike …

Robin Grapa and Patty Laatsch are hiking across the country to benefit bone marrow disease patients and families everywhere! They started in Delaware this past February and have – thus far - hiked through Maryland, West Virginia, Ohio, Indiana, Illinois, Missouri, Kansas, and are almost entirely through the state of Colorado.

That is nearly 3,448 miles, which equates to 7,292,176 STEPS – can you imagine?

“Our Hike” is the idea of Robin, an aplastic anemia patient in remission. Her plan was to do something that would not only raise awareness for a disease so many people know nothing about, but to also raise money to benefit the many patients and their families who continue to endure on a daily basis. Robin and her family know, first hand, what it is like to be faced with a disease they not only have never heard of before, but could barely pronounce. They not only know the pure fear that immediately hits, but they also know the comfort found when the answers and support they so desperately needed in order for a better understanding of what they faced was received. AA&MDSIF provided them this support and helped them along their way.

It is not only the commitment of Robin and Patty that has been undying to this cause, but their family as well. Patty’s husband and Robin’s father – Keith – acts as the “hub” of the operation at home in Wisconsin, working full-time, gathering their needs for the trail and sending goods off to them at their mailstops, among many other things. Adam, Robin’s husband, continues to work full-time maintaining their household expenses on only one income, while Robin is hiking for our cause. Their hometown in Wisconsin hosts fundraisers at various intervals along the trail; and their extended family – aunts and uncles – have hosted events in their perspective hometowns, as well.

In addition, many incredible families and friends along the trail have stepped in to help. Robin and Patty have been hosted for overnights in hotels and welcomed into so many friendly homes, had lunches and dinners with new and old friends, trail angels have brought them cold water and refreshments, strangers even have offered their homes in both good weather and bad – a warm bed and nice hot shower for those weary feet and aching muscles! They continue to receive the kindness of those who are not strangers to the disease, and those who are.

The goal of Our Hike is $1,000,000. Thus far, they have raised nearly $96,000. It is now your turn to take steps to help THEM. Ask your friends, your family, your colleagues, your neighbors all to make donations. By the end of their trek across country, Robin and Patty will have taken 10,412,160 steps for AA&MDSIF, for fellow patients, fellow caregivers, fellow parents, fellow sisters and brothers, for all of YOU touched by bone marrow disease. It is up to you now to take the steps necessary to push Our Hike to its goal of $1,000,000.

Please - Donate Now.

Learn more about Our Hike at the information table in the Exhibit Area.
As you make this donation, keep in mind the extent of what Robin and Patty are doing for patients and their families throughout the country. Imagine hiking – BY FOOT – over 5,000 miles across the United States on behalf of patients, families, and caregivers battling bone marrow disease in order to spread awareness and raise funds to fight this disease. It is truly incredible. Show your appreciation for what they are doing for those who can’t – DONATE NOW.

Print Your Name: ________________________________
Address: ______________________________________
City: __________________________________________
State: _________________________________________
Zip: __________________________________________
Daytime Phone: (_____) __________________________
Email Address: _________________________________

Enclosed is my tax-deductible gift of:
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PAYMENT OPTIONS:
☐ Make your check, money order or traveler’s check payable to: Aplastic Anemia & MDS International Foundation, Inc.

☐ By Credit Card:
  ☐ VISA  ☐ MasterCard

  Account #: _________________________________
  Exp. Date: _________________________________
  Signature: _________________________________

This gift is made in honor of OUR HIKE

SPONSOR THE HIKERS

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<tr>
<th>Per Hiker</th>
<th>Robin</th>
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<tr>
<td>$101.14 ($ .02 per mile)</td>
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<td>$252.85 ($ .05 per mile)</td>
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Total Donation Amount = $________________________________

☐ I have enclosed my company’s Matching Gift Form in order to increase my giving to AA&MDSIF.

☐ I am interested in obtaining information regarding the AA&MDSIF Planned Giving Program. Please contact me with information on ways of giving by means of wills, trusts, stock, life insurance, real estate, etc.

______________________

All annual donations totaling $500 or more received by December 23 will be acknowledged in the Annual Report and End-of-Year Thanks published in our Winter Newsletter.

______________________

AA&MDSIF is supported through individual contributions and is a non-profit charitable organization as described under the Internal Revenue Code, Section 501(c)(3).

If paying by credit card, FAX this form to (410) 867-0240. If paying by check, mail check along with this form to the above address. Or you may wish to donate directly online at www.aamds.org.
We fight back.

We are Pharmion. Improving the lives of patients and their families is at our core. Every day we work with doctors, researchers, regulators and organizations all over the world to stop the hematologic diseases that ravage lives. We are dedicated to creating innovative drug therapies that fight back against these diseases. We are proud to partner with the AA&MDS International Foundation, and we salute and support its tireless efforts. After all, we’re in this together.
Alexion Pharmaceuticals
is a proud sponsor of the AA-MDS Annual Patient & Family Conference

Alexion Pharmaceuticals is committed to research and expanding awareness about PNH

To learn more about PNH, visit:

www.PNHSource.com
A new educational resource for PNH

www.alexionpharm.com

ALEXION Pharmaceuticals

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Is Proud to Support the
the AA&MDSIF
Family & Patient Conference!
We are the oldest and largest patient advocate and support organization for bone marrow diseases providing life-saving hope, knowledge, and support to hundreds of thousands of patients and their families around the world.

**We are your partners in care.**

We give you the expertise of our distinguished global team of medical experts, scientific researchers, doctors, nurses, counselors, information specialists, government agencies, political advocates, pharmaceutical companies, patients, caregivers, families, and Board of Directors and staff.

**HOW WE HELP**

Patients, caregivers, families, and health care professionals can benefit from these FREE services:

- **Educational Materials** explain the diseases, treatment options, research updates, clinical trials, patient rights, managing treatment decisions, and handling emotional issues.

- **Patient Information Department** answers your questions with up-to-date and reliable information from medical experts.

- **Emotional Support** provided by toll-free hotline and email correspondence.

- **Global Network of Volunteers** composed of patients, families, and caregivers share physical, emotional, and coping experiences with other families.

- **Clinical Trials Information & Referral Department** provides one-on-one education and referrals; study listings, including locations, doctors’ contact information, and protocol descriptions; and financial assistance for treatment-related travel.

- **Quarterly Newsletters** and **Monthly E-Bulletins** feature medical updates, research articles, victory stories, helpful resources, and AA&MDSIF activities.

- **Annual Patient Conferences** gather hundreds of patients, caregivers, families, medical researchers, and health care experts to discuss advances in research, treatment options, lifestyle changes, coping strategies, and emotional aspects of illness.

- **Voluntary Patient Registry** collects patient statistics to help medical researchers better understand, prevent, and cure bone marrow diseases.

- **Medical Research** studies located around the world are financially supported by the AA&MDSIF in hopes of finding effective treatment and a cure.

- **International Bone Marrow Failure Scientific Symposium** provides researchers from around the world with a forum for presentation of their work and the opportunity to work together on bone marrow diseases.

- The **AA&MDSIF Medical Advisory Board** composed of distinguished medical experts, advises on research funding, patient information, and educational materials.
Emergency Phone Numbers ...

Summit Medical Center
5655 Frist Boulevard
Hermitage, TN
General: (615) 316-3000
Blood Bank: (615) 316-3882
American Association of Blood Banks Accreditation

~ 10 minutes from hotel

Vanderbilt University Medical Center
1211 22nd Ave. South, in the downtown/Vanderbilt area
Nashville, TN
General: (615) 322-5000
Hematology/Oncology: (615) 322-4967
Emergencies: (615) 322-4967

~ allow 1 hour due to road construction

Walgreen’s Pharmacy
518 Donelson-Elm Hill Pike
Nashville, TN
Store: (615) 883-4706
Pharmacy: (615) 883-5108
Hours: 24/7

~ 2 minutes from hotel

Yellow Cab
Taxi service: (615) 256-0101
Call Our
Patient Information Department
for the Answers You Need

Aplastic Anemia & MDS International Foundation (AA&MDSIF) provides patients, caregivers, families and healthcare professionals with the most current information and answers they are looking for on bone marrow disease. Our patient information specialists are available by calling our Toll Free Helpline - (800) 747-2820 - within the United States, Monday through Friday, 8:30am to 5:00pm (ET), or email help@aamds.org. Callers outside the United States should dial (410) 867-0242.

Contact us for a complete listing of the patient services we offer.

Fighting Bone Marrow Disease Through Patient Support & Research Since 1983.

(800) 747-2820  www.aamds.org