AA&MDSIF at ASH 2011: News for Patients, Products for Professionals!

The American Society of Hematology (ASH) 53rd Annual Meeting was held December 9-13, 2011 in San Diego, California. With over 20,000 attendees, this high-profile event is the world’s largest professional meeting of hematologists/oncologists and is where many major findings are first announced to attendees, the larger medical and scientific community, and the media.

At ASH 2011, AA&MDSIF premiered new programs and products of interest to professionals and patients alike.

New Interviews with Important Information

At the 2012 ASH meeting, AA&MDSIF worked for the first time with Andrew Schorr, host of Patient Power (www.patientpower.info) to interview three leading experts about the most important information being presented.

He interviewed Dr. Mikkael Sekeres on MDS, Dr. Carlos de Castro on PNH, and Dr. Neal Young on aplastic anemia. These interviews are available now on the Patient Power media player (www.patientpower.info/AAMDS).

More than 100 researchers from around the world gathered on March 22nd and 23rd for the third Bone Marrow Failure Disease Scientific Symposium in Bethesda, Maryland. Experts joined with young investigators to learn about the key areas of current research and to explore the most promising directions for the future.

Highlights included:
- New discoveries of genetic mutations in MDS that may lead to better diagnosis, prognosis, and treatment
- Advances in hematopoietic cell transplantation for bone marrow failure diseases
- New drugs and drug combinations currently in clinical trials
- Developing treatment options for aplastic anemia, MDS, and PNH

Symposium Co-Chair, Neal Young, MD, of the National Heart, Lung, and Blood Institute at NIH concluded that, “It was a conference of extraordinary science and enthusiasm. Excellent basic science and major new advances in clinical care were presented by the world’s experts. The lively and close interactions of the participants were especially valuable in fostering future work and encouraging young investigators. I think it was the best bone marrow failure conference ever!”

Comments from Other 2012 Symposium Participants

“This meeting is an opportunity for world leaders in MDS to get together in a much more intimate setting than most large conferences can afford.”

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Some of you may know that during my 42 years of service in nonprofit organizations, a significant amount of that time involved executive, board, and management development; helping groom leaders for the nonprofit world. This required keeping abreast of the latest in management thinking and practice.

Nearly 20 years ago, James Collins and Jerry Porras wrote a bestselling book entitled *Built to Last*. In it, they describe many elements that contribute to the long term success of organizations. One of those elements is to “reject the tyranny of the OR and embrace the genius of the AND”. It doesn’t mean to try to do everything, but rather to acknowledge that you can do two seemingly opposite things well. This is especially true if you view the two things as complementary.

When we developed our most recent strategic plan, we looked at needs as well as opportunities and found some very interesting and complementary ways to serve more patients and families, while also serving more health professionals. And we could serve everyone better.

Offering new programs for health professionals serves to make more community hematologists, oncologists, and their staffs more aware of aplastic anemia, MDS, and PNH and more aware of AA&MDSIF. If they knew more about us, they can recommend us and our services to more patients, families and caregivers. So we developed a toolkit for community based hematologists/oncologists which includes professional education materials, a mobile app, AND patient education materials to share with those they are treating. These physicians are a particularly important group since they treat approximately 50% of all aplastic anemia, MDS, and PNH patients. At your next visit, ask your treating physician if he or she has our toolkit. If not, they can request one online at www.AAMDS.org/mdstoolkit.

A second area where AND is better than OR is where to concentrate our other educational efforts. For years, we have focused our materials, programs, conferences, and webinars for patients, appropriately so, since they— you— are our top priority. The group we were missing, the group most critical with living well, is caregivers. They are the spouses, family members, and friends who are there each and every day to help, to support, to love, and to care for the patient. Sometimes we have been so focused on patients that we missed the obvious.

So this year, we are making a special effort to address the needs and concerns of caregivers at our conferences, in several new webinars, in our publications and on our Web site.

Yes, we can focus on research AND advocacy; professional education AND awareness. The more times we can embrace the ANDs, the more services we can provide; the more people we can serve.

Stay well!

John M. Huber
Executive Director
AA&MDSIF Active at ASH 2011 (continued from cover)

- **MDS:** Several promising new developments in MDS diagnosis and treatment were reported at ASH. In our interview with Dr. Mikkael Sekeres of Cleveland Clinic, he was excited about the important progress being made in clinical trials for combinations of drugs for some MDS patients. He also noted that researchers have identified previously unknown genetic abnormalities that can better predict patients’ prognoses and their responses to drug therapy.

- **PNH:** Dr. Carlos de Castro of Duke University noted two important findings reported at ASH on pediatric PNH. Researchers comparing clinical characteristics of pediatric PNH patients compared to adult PNH patients found no significant differences. Also, data shared at ASH will likely lead to the approved use of eculizumab (Soliris®) for pediatric patients in the future.

- **Aplastic Anemia:** Dr. Neal Young from NIH National Heart, Lung and Blood Institute (NHLBI) reported that several studies presented at ASH focused on the prognostic importance of short telomeres and how telomeres can predict patients’ responses to types of therapy for aplastic anemia.

  In addition, the drug alemtuzumab (Campath®) is showing good potential as another immunosuppressive agent. A new drug called eltrombopag (Promacta®), which is already approved by the Food and Drug Administration (FDA) for ideopathic thrombocytopenia (ITP), is showing promising results as an oral agent that would work directly on stem cells to treat aplastic anemia.

  Dr. Young is encouraged by the progress being made and believes new lab data will help physicians understand even more about how to best target treatment for individual patients.

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### New Resources for Healthcare Providers

AA&MDSIF is pleased to announce the launch of the Treating MDS Toolkit, a free resource developed to provide healthcare providers with materials to help communicate with and support their patients with MDS.

The toolkit was developed in response to the results of a survey conducted by AA&MDSIF and led by Dr. Mikkael A. Sekeres, summarized in the journal *The Oncologist* in 2011 (*Perceptions of Disease State, Treatment Outcomes, and Prognosis Among Patients with Myelodysplastic Syndromes: Results from an Internet-Based Survey*. Sekeres, et al. The Oncologist 2011; 16:904-911). The survey found that many MDS patients lack knowledge of MDS disease basics and are unaware of the severity of the disease. As a partner in patient care, AA&MDSIF designed this toolkit to help physicians share necessary information with their patients efficiently and effectively.

The toolkit contains an array of patient information sheets that can be distributed to patients at the physician’s discretion based on their level of knowledge of the disease, the frequency of their office visits, and their individual diagnosis. Patient information sheets topics include: *What is MDS, Knowing your MDS Risk Level, Understanding the Symptoms of MDS, Treatment for Lower-Risk MDS, Treatment for Higher-Risk MDS, Managing the Side Effects of MDS Therapy, Staying on Therapy and Preparing for Office Visits.*

The toolkit also has a counseling guide which explains how to use it as well as information about the MDS Patient Survey, including a reprint of the article published in *The Oncologist*. In addition, AA&MDSIF patient education booklets and an order form are provided so healthcare providers can order more materials for their office as necessary.

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Bone marrow failure diseases are complex. You need to learn all you can about your condition and the available treatments. Staying well-informed helps you be a powerful player on your healthcare team. It allows you to educate family and friends, get more out of office visits, make choices about your treatment, and keep an eye on your care. Today, it’s easier than ever to learn about your disease and treatment options. You can find information from many print resources including books, brochures, newspapers, medical journals, magazines, and—of course—the Internet.

Your first source of information may be your healthcare team. This may include your physician, a nurse or nurse practitioner, research assistant, resident, or physician assistant. Ask questions and request materials.

**Be sure to use AA&MDSIF services.**

**Request a patient information packet from AA&MDSIF.**
You can order a free information packet from AA&MDSIF right on our Web site (www.AAMDS.org/Info), or you can call us at (800) 747-2820.

**Read all of the relevant sections of our Web site.**
Our Web site (www.AAMDS.org) has a wealth of information on aplastic anemia, MDS, and PNH.

**Take advantage of the Online Learning Center.**
You’ll find video presentations, interviews with medical experts, and interactive learning modules. You can even take part in a live webinar. Visit the Online Learning Center at www.AAMDS.org/Learn.

**Talk to the AA&MDSIF patient educator.**
Call us at (800) 747-2820, option 1, with your questions.

**Attend our patient and family conferences.**
AA&MDSIF patient conferences are great places to learn more about your disease. You can hear from leading medical experts, meet and speak with other patients, and pick up materials. In 2012, AA&MDSIF conferences are being held in six metropolitan areas around the U.S. See www.AAMDS.org/Conferences for information and registration details.

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Explore other reliable resources of medical and health information, and your treatment options.

**Visit the National Library of Medicine Web site.**
At www.nih.nlm.org, you’ll find detailed information on many diseases. They have a simple search tool on the main page. From this site you can also use MedLinePlus, a Web site for patients and their families and friends maintained by the National Institutes of Health.

**Thinking of participating in a clinical trial?**
Clinical trials are used to test new treatments before they’re made available to the public. If the new treatment proves to be better than the current treatment, the new treatment can then be approved for general use by the U.S. Food and Drug Administration (FDA). You may want to consider taking part in a clinical trial, also called a research study. These studies can offer good treatment options for some people with bone marrow failure diseases like aplastic anemia, MDS, and PNH. You can directly access this information at www.clinicaltrials.gov.

**Consider your preferences and make a plan that best suits them.**
When the time comes to make a decision about treatment, use reliable information—along with input from your healthcare providers, family, and friends. Weigh the pros and cons of each treatment. Think about how well it might work, side effects, and costs. Consider how it might affect your lifestyle. If side effects are tough to handle, your doctor may be able to prescribe a medicine to lower the side effects. Always talk to your doctor before you stop any medicine.
IN FUTURE ISSUE OF THE INSIDER
Standing Up for Your Health -- Pillars 3 through 5

3. Make the most of every office visit.
5. Build a strong personal support team
Learn more at www.AAMDS.org/StandingUp

Scientific Symposium (continued from cover)

“Great science, unbiased, noncommercial. Great venue. Good timing (doesn’t conflict with other meetings).”

“I love that you support junior faculty and encourage them to attend the meeting. Arguably, they (me) get most out of attending.”

Patient Summary and Live Interviews
In May 2012, AA&MDSIF will publish a Symposium Summary for Patients in both print and online formats. This lay language publication will summarize the symposium presentations, with emphasis on what the research means for patients.

Certain speakers were also interviewed at the symposium to provide highlights of their presentations and discussions of important progress in bone marrow failure disease research. These will be available sometime in late spring on our Online Learning Center, (www.AAMDS.org/Learn)

Several current and former AA&MDSIF research grantees participated in the symposium as speakers and as poster presenters.

• Matthew Walter, MD, of Washington University in St. Louis, a 2005 AA&MDSIF grantee, credited his grant from AA&MDSIF as the start of his research into the genomics of MDS. Dr. Walter and his colleagues recently published the results of their significant work in the New England Journal of Medicine.

• Lisa Minter, PhD, of the University of Massachusetts Amherst, received two research grants from AA&MDSIF that “made her career” in studying aplastic anemia. In addition to being a rising young investigator in bone marrow failure disease, Dr. Minter lost a daughter to aplastic anemia several years ago and became absolutely committed to studying this disease and to the mission of AA&MDSIF.

The videos of their comments are available on our YouTube channel, www.youtube.com/user/AAMDSIF.

The 2012 Scientific Symposium was supported by the National Heart, Lung, and Blood Institute and the Office of Rare Disease Research of the National Institutes of Health, several pharmaceutical companies, and the Edward P. Evans Foundation. The next Scientific Symposium is already being planned for March 2014.

Patient Guide Available from AA&MDSIF
You can also order a copy of our Standing Up for Your Health patient guide.

It features much of the same content seen at www.AAMDS.org/StandingUp and is organized by the five pillars of Standing Up for Your Health.

To get your copy, call (301) 279-7202 or (800) 747-2820, x116 or email harper@aamds.org

Current and past AA&MDSIF research grant recipients (left to right): Dr. Lisa Minter, Dr. Seth Corey, Dr. Cristian Bellodi, Dr. Kim-Hein Dao, Dr. David Araten, Dr. Jaroslav Maciejewski, Dr. Antonio Risitano.
AA&MDSIF Active at ASH 2011 (continued from page 3)

Satellite Symposium a success
AA&MDSIF and Cleveland Clinic co-sponsored the symposium “Successes in Bone Marrow Failures” at ASH to present the most up-to-date diagnostic and treatment options for aplastic anemia, MDS, and PNH. This event featured an internationally renowned faculty and drew nearly 300 hematologists and other allied health professionals.

AA&MDSIF now involved with the following cooperative efforts:

Global Forum for MDS Patient Groups
AA&MDSIF Executive Director John Huber co-chaired the inaugural meeting of the Global Forum for MDS Patient Groups along with Sophie Wintrich of the MDS-UK Patient Support Group. The purpose of this forum is to identify common needs of MDS patients around the world and to share ideas and resources among the participating groups to address these needs.

A survey conducted among the forum member organizations indicated that the major issues affecting patients and caregivers were access to diagnosis and availability of treatment. The major concerns of the MDS patient groups were increasing MDS awareness and limited resources, both in terms of financial support and people. The Global Forum will continue communicating through email, periodic conference calls, and live gatherings when possible.

Life Beyond Limits Campaign
A new global campaign entitled “Life Beyond Limits” was announced during ASH with the goal of improving the treatment of older cancer patients. “Life Beyond Limits” aims to raise awareness of the benefits of equal access to treatment regardless of age and to empower others in their cancer journey.

The official launch was held as part of a photo exhibit “Facing MDS: When Every Moment Counts” by internationally acclaimed photojournalist, Ed Kashi, at the San Diego Museum of Art. The exhibit features portraits and self-authored stories of six MDS patients from around the world.

Kirby Stone, an MDS patient and active AA&MDSIF volunteer from Ohio, was one of the patients featured in the photo essay.

“Life Beyond Limits” is being driven by a steering committee of MDS experts with a broad range of experience in medicine, research, patient and family relations, and personal experience with MDS. Members of the committee include John Huber, AA&MDSIF Executive Director, and Dr. Valeria Santini, Associate Professor of Hematology at the University of Florence Medical School in Italy, and an AA&MDSIF Medical Advisory Board member.

Research Presented at ASH Available in Multiple Formats
Following up our attendance at ASH 2011, AA&MDSIF produced webinars with the latest information reported at ASH on aplastic anemia, MDS, and PNH. We asked several experts to present new information from the ASH annual meeting related to aplastic anemia, MDS, and PNH, in an easy to understand format for patients, their families and friends.

Aplastic Anemia
Aplastic Anemia Clinical Update for Patients, presented by Pamela Becker, MD, PhD

MDS
Emerging Treatments and New Protocols for MDS Therapy, presented by Mikkael A. Sekeres, MD, MS, and David Steensma, MD.

New Strategies for Diagnosing and Classifying MDS in 2012: Can This Change Treatment Outcomes? Presented by Timothy Graubert, MD

PNH
PNH Clinical Update for Patients presented by David Araten, MD

These webinars are now posted on the AA&MDSIF Online Learning Center (www.AAMDS.org/Learn).

ASH Summary Booklets on Aplastic Anemia, MDS and PNH
In addition to the webinar presentations and accompanying slides, we have created three easy-to-read ASH abstract summary booklets summarizing key research findings from ASH 2011. These booklets focus on information we believe will be most important and helpful to you.

ASH abstract summary booklets on aplastic anemia, MDS and PNH are now available online for convenient downloading at www.AAMDS.org/ResearchSummaries.

If you prefer, you can request print copies of the MDS and PNH summary booklets (aplastic anemia summary is available as download only) to be mailed to you by contacting harper@aamds.org or by calling (301) 279-7202, x116.
“As healthcare providers, it’s our responsibility to educate our patients and their caregivers about this serious disease,” said Mikkael A. Sekeres, MD, MS, Cleveland Clinic. “We play a critical role in their disease management and by offering the Treating MDS Toolkit as a resource, AA&MDSIF is really showing their commitment to their partnership in patient care.”

According to John Huber, AA&MDSIF Executive Director, “This toolkit is a direct result of listening to our patients and responding to their needs. A diagnosis of MDS is life-changing and difficult to comprehend, and this new resource will facilitate communication between healthcare providers and MDS patients and their caregivers.”

To order a free toolkit, health care providers should visit www.AAMDS.org/treating-mds-toolkit. Materials can also be downloaded online. Patients are encouraged to be sure their treating physician is aware of this free resource for healthcare professionals.

AA&MDSIF Mobile App for MDS Risk Classification and Stratification Now Available

This free mobile app for smartphones is now available for Android, BlackBerry and iPhone platforms. Features include:

- Medical calculators for IPSS, WPSS, FAB and WHO
- Resource section including links to NCCN Guidelines, AA&MDSIF Web site, journal articles and CME programs
- Extensive FAQ section with answers by MDS experts

Visit www.AAMDS.org/mobileapp for more information.

AA&MDSIF 2009-2011 Grantees Present Final Reports

All three of the two-year research grants awarded by AA&MDSIF in 2009 have been successfully concluded with final reports submitted.

Regis Pefafault deLatour, MD, PhD, Hospital Saint Louis, Paris, France

Funded by the PNH Research Foundation

The Role of Unfolded Protein Response in PNH

The initial scope of this grant concerned the potential role of the Unfolded Protein Response (UPR) in Paroxysmal Nocturnal Hemoglobinuria (PNH). PNH is caused by a gene mutation resulting in “unfolded” proteins in deficient cells. This study found that these deficient cells are more susceptible to proteasome inhibitors, or drugs that block the action of proteasomes (cellular complexes which breakdown proteins and contribute to red cell destruction in PNH).

Dr. deLatour and his colleagues also conducted important research in the field of severe aplastic anemia (SAA) with or without PNH. They assessed a new lymphocyte subset, called Th17 cells, in patients with aplastic anemia. The role of this new subset was so far unknown in aplastic anemia. Using marrow cells from both aplastic anemia patients and mouse models, these researchers demonstrated that Th17 immune responses contribute to aplastic anemia pathophysiology, especially at the early stage during disease progression.

They also assessed the potential role of Th17 cells in graft versus host disease (GvHD), a complication after hematopoietic stem cell transplantation (HSCT). Dr. de Latour assessed the role of Th17 cells in GvHD, and found that regulatory T cells normally able to regulate the immune system activity were underrepresented compared with T cells (including Th17 cells) responsible for GvHD.

The research team also looked at the difficult question of HSCT in PNH. This study first confirmed, on a large cohort of PNH patients, that HSCT using related or unrelated donors is a valuable curative option for PNH complications. It also suggested, however, that HSCT should not be the first option in case of thromboembolism in PNH, because of the potential toxicity of such treatment and of the actual treatment by Eculizumab.

“I am grateful to AA&MDSIF and the patients who gave me the great opportunity to develop such important work with experts in the field of bone marrow failure,” said Dr. deLatour.
Kazuhiko Ikeda, MD, PhD
Fukushima Medical University, Fukushima, Japan
\*PNH Research Foundation Grant

Investigations into the ‘Two-Hit-Hypothesis’ and the Role of HMGA2 Overexpression.

AA&MDSIF research grantee, Kazuhiko Ikeda, MD, PhD, moved from the U.S. to accept a faculty position at Fukushima Medical University in Japan early in 2011, where his work was disrupted by the significant earthquake and subsequent nuclear plant accident. Although Dr. Ikeda and his colleagues needed some time to recover from the disaster, he is pleased to report that their medical centers and research facilities have returned to full operation and he recently restarted his grant study.

To date, Dr. Ikeda and his colleagues have investigated the molecular pathways that cause a growth advantage of PNH cells caused by a particular gene mutation. They expect that their findings may lead to a better understanding of why PNH develops and possible therapies to inhibit the growth of PNH cells.

“I am most grateful to Dr. Monica Bessler and Dr. Phillip J. Mason at the Children’s Hospital of Philadelphia for their valuable and faithful support when I was conducting my research there, and to AA&MDSIF for the generous extension of the funding after the disaster here in Japan,” said Dr. Ikeda.

Archibald S. Perkins, MD, PhD
Department of Pathology and Lab Medicine, University of Rochester Medical Center
\*AA&MDSIF Harold Spielberg Research Grant

Development of targeted therapies for 3q26-positive MDS

Dr. Perkins and his colleagues have been studying the gene located at chromosome 3, in a region called band q26. Rearrangement of this particular gene, termed EVI1, is associated with a particularly poor prognosis for MDS. His research was aimed at trying to develop a novel type of drug therapy that will specifically block the activity of the EVI1 protein. Such a targeted therapy, if effective and without toxicity, could represent a new way to treat MDS.

Dr. Perkins reports that developing a targeted therapy for EVI1 presents a particularly daunting challenge, since the protein lacks the features that one likes to see in typical drug targets. Most drug targets have “pockets” that are critical for activity, and the drugs typically bind to these pockets and thereby inhibit the protein’s activity. EVI1 possesses no such pockets. However, his research has shown that EVI1 binds specifically to DNA, and his team has developed a compound that binds specifically to the DNA binding site of EVI1 rather than to the EVI1 protein itself. This represents a novel approach to inhibiting protein function, and may be applicable to the inhibition of other proteins of this type.

“Our results to date are promising,” notes Dr. Perkins. “We have shown that our compound definitely has activity in inhibiting EVI1 function in the test tube; however, its activity in inhibiting EVI1 in living cells still needs more work, which we are currently working on.”

“We wish to thank AA&MDSIF for supporting us on this very promising drug development project,” said Dr. Perkins.

Gregory A. Abel, MD
Dana Farber Cancer Institute
\*AA&MDSIF Research Grant including support from the Madden family in memory of Mary Pat Madden Greishaber and the MacGillvray family in memory of Erwin Umbach

Developing a Disease-Specific Measure for Quality of Life in Patients with Myelodysplastic Syndromes (MDS)

Over the past year, we have initiated the process of developing a disease-specific measure of quality of life for patients with myelodysplastic syndromes. The most significant part of this effort was to host an evening focus group for 32 MDS patients, caregivers and healthcare providers in April 2011 at the Dana-Farber Cancer Institute.

The purpose of this focus group was to hear directly from MDS patients and their health-care providers (inpatient and outpatient nurses, physician assistants, social workers, and physicians) regarding the factors that are most important to the quality of life of MDS patients and how we could best capture those factors in a questionnaire. Interestingly, participants rated fatigue, emotional health, and uncertainty as the three most important factors. Moving forward, we plan to use the data obtained from the focus groups to create an MDS-specific quality of life survey which we will then pilot and refine with another cohort of MDS patients.
Emerging Role of p53 Translation Control in Hematopoietic Stem Cell Quiescence and Differentiation

During this first year of funding, we have made important progress towards carrying out the specific research aims outlined in the original research proposal. Employing special transgenic animals, we have shown that reductions in the amount of DKC1 affect the number of pluripotent hematopoietic stem cells in the bone marrow. At present, we are investigating how these molecular defects affect the production of the p53 protein within the HSC population directly in the animals.

We are using a novel approach to dissect the molecular defects underlying impaired p53 production in transgenic mice and human X-DC patient cells. In this respect, we have made substantial advances in generating all the biochemical tools necessary to perform the experiments proposed. It is our expectation that results from these studies may possibly lead to the identification of new targets for therapeutic treatment.

We have also made substantial progress in delineating the basis for the molecular defects present in a group of human X-DC patient cells carrying distinct point mutations in the DKC1 gene. Interestingly, we have demonstrated for the first time that ribosomes are defective in these X-DC mutant patient cells and we are currently testing whether these mutations impair p53 production and activity.

In the next year of funding, we intend to expand these studies to gain more insight into the molecular mechanisms underlying hematological abnormalities such as bone marrow failure and MDS.

The primary treatment for patients with idiopathic aplastic anemia who are ineligible for bone marrow transplant is the use of antithymocyte globulin (ATG). Relapsed and refractory cases of aplastic anemia can occur even after ATG treatment. Alefacept® is a novel immunosuppressive agent that targets the CD2-LFA3 pathway important in various T cell functions, especially T cell activation. The occurrence of relapsed or refractory cases of aplastic anemia coupled with the side effect profile of ATG prompted this study that investigates the utility of Alefacept® in aplastic anemia.

This Phase 1/2 study has now been fully IRB approved at the Cleveland Clinic and is open for enrollment. A total of four informed consents have been sent to four patients who have expressed interest in participating in the study. One patient has been screened as of June 13, 2011 and if eligible will start treatment on June 20, 2011. A second patient will be screened in two to three weeks. All correlative studies originally incorporated in the study to help understand the effects of Alefacept® in T cell function in aplastic anemia and the role of CD2-LFA3 pathway in aplastic anemia have been tested and prepared. The correlative studies include PNH flow cytometry, immunodeficiency panel, cytokine profile and CD2 receptor saturation.

Loss of chromosome 7 (monosomy 7) occurs very frequently among adult and pediatric patients with myelodysplastic syndromes (MDS) or bone marrow failure syndromes that progress to myelodysplasia. Monosomy 7 cells express a defective receptor for the blood growth hormone G-CSF. With the support by AA&MDSIF, I found that the defective receptor deregulates the transcription factor JunB, resulting in abnormal blood formation. During the second year of my study, I will further investigate the role for JunB using a mouse model, which will help to identify the biochemical changes that make the monosomy MDS cells different from normal blood cells. By studying the components that make a monosomy 7 blood cell diseased, we can develop new types of therapies to correct them which are more effective and less toxic than current bone marrow transplantation.

Since receiving his grant from AA&MDSIF in 2010, Dr. Futami accepted an assistant professorship position at the University of Tokyo Institute of Medical Science, but he is continuing to consult with and supervise his colleagues at Northwestern University who are continuing the second year of his research.

The blood samples obtained during the screening visit of the first patient are in the process of being analyzed. Now that the study is fully open, we have received interest from additional centers who have expressed willingness to send patients who have relapsed/refractory aplastic anemia for participation in the clinical trial. We hope to progress quickly in the next few months.
2012 Regional Patient and Family Conferences
LIVING WITH APLASTIC ANEMIA, MDS OR PNH

Building on the success and strong attendance at the 2011 Patient and Family Conferences, the AA&MDSIF conference series for 2012 comes to six more metropolitan areas.

MARK YOUR CALENDAR NOW FOR THE LOCATION NEAREST YOU!

★ March 24  WASHINGTON, DC
★ April 21  LOS ANGELES, CA
★ May 19  ATLANTA, GA
★ July 28  NEW YORK CITY AREA
★ September 22  DALLAS, TX
★ October 20  CHICAGO, IL
★ June 16  SEATTLE, WA

In addition, AA&MDSIF is co-hosting a conference with the Fred Hutchinson Cancer Research Center on June 16 in Seattle, WA. This conference format will differ from the other six conferences. See Seattle conference Web site for agenda details.

AA&MDSIF Patient and Family Conferences

Have you ever met another person with aplastic anemia, MDS or PNH? Seven out of ten conference attendees had never met another person diagnosed with aplastic anemia, MDS or PNH until they connected with them at one of our conferences.

If you haven’t attended one of our Living with Aplastic Anemia, MDS or PNH patient and family conferences, mark your calendar now to attend! Patients will have the opportunity to attend a conference within just a few months of their diagnosis. The one-day format makes it easy for family members to participate at a time that’s convenient to school and work schedules.

Attendees will find several enhancements to the 2012 conference program. These changes, based on input and suggestions received from attendees and presenters, are designed to increase the value of these conferences. Although the core program remains the same, our 2012 programs will focus on providing even more opportunity for participants to connect and share, along with some specific programming just for caregivers.

Why Should You Attend One of Our Conferences?

- **Hear from leading experts.** Learn more about your diseases, current treatments, and emerging therapies.
- **Explore a variety of topics** on living well and improving your quality of life.
- **Get your questions answered.** Plenty of time will be provided in every session.
- **Learn skills and strategies** that can help you stand up for your best medical care and become a more powerful patient.
- **Meet other patients and family members** and get connected to a support network.
- **Participate in our Celebration of Hope** and leave with a sense of hope and a positive outlook for the future.
- **Understand how AA&MDSIF can help** you through this life-changing experience.
- **Like any AA&MDSIF live event**, registration is free and includes meals and all materials. However, advance registration is required. For more information, and to register, visit www.AAMDS.org/Conferences.

For more information
www.AAMDS.org/Conferences
Meet more patients and families, take home more expert information!

When you attend one of our conferences, here is what you can expect:

Answers to your medical and treatment questions
The morning sessions include concurrent tracks on advances in research and treatment methods for each disease—aplastic anemia, MDS, and PNH. During these sessions, participants will have unprecedented access to the nation’s leading bone marrow failure disease experts, with plenty of time for everyone’s questions to be answered.

Sessions on Living Well
The afternoon will include a series of Living Well sessions (this will vary by location) on topics including managing fatigue, complementary and alternative medicine, nutrition, and exercise, coping skills for maintaining emotional health, and survivorship. Three sessions will be offered at each location.

New for 2012!
Support forums to help you connect, share, and learn from others
These professionally-facilitated support sessions will bring together patients and family members with similar issues and concerns. They provide plenty of time for talking, sharing, comparing, and hearing how others are coping with the challenges of living with a bone marrow failure disease. Sessions are broken out by disease type. A special session for caregivers will also be included. Sessions for parents of pediatric patients will be provided at our programs in Los Angeles, Atlanta, and New York.

Hope for the future
Each conference concludes with a powerful and uplifting Celebration of Hope. This joyous gathering celebrates life and friendship. The program includes a survivor spotlight story, highlighting the struggles and triumphs of one of the many long-term bone marrow failure disease survivors who is successfully living with and beyond their illness. We also include a special presentation, including pictures, quotes, and comments provided by conference participants in the weeks leading up to each conference. Be sure to come and celebrate your life and your courage with us.

Here’s what conference attendeees had to say about their experience:

I was impressed with the conference, the staff, volunteers, the warmth and friendliness of everyone. It was a well-thought out conference.
Gayle, aplastic anemia patient

I really enjoyed your conference. It answered a lot of questions for my daughter and me. We left your conference feeling great. WE LOVED IT! Keep up the good work because you do make a difference.
Diana, mother of aplastic anemia patient

Thank you for all your hard work in putting these conferences together for us. It was an amazing and wonderful experience for me, and I will make sure to attend next year.
Leslie, MDS patient

I have attended many, many conferences in the last 50 years on many subjects, and this was the most organized and informative of them all!
Wayne, MDS patient

Great job! Very supportive and hopeful atmosphere. Wonderful presentations of practical and meaningful information. Please receive our sincere thanks for your vital and life-giving work.
Lon, PNH patient

Make plans to attend…register now!
Our 2012 conferences will provide answers, support, and hope for every patient, family member, and caregiver whose lives have been touched by bone marrow failure disease. If you’ve never attended one of our conferences, you won’t want to miss this informative, inspirational, and uplifting event.
The Aplastic Anemia & MDS International Foundation Online Learning Center (OLC) is a comprehensive patient education portal with a wealth of different multi-media learning opportunities. All OLC content is free and available to anyone with access to a computer and a high-speed Internet connection.

Understanding the Different Formats
Our Online Learning Center presentations are provided in four formats – webinars, webcasts, interviews with experts, and interactive modules. In this issue of the Insider, we’ll review the interactive modules, which can be directly accessed at www.AAMDS.org/Interactivemodules.

Interactive modules are online learning tools. Information is presented in an easy-to-understand format, using graphics, animation, and audio. Currently, the OLC contains interactive learning modules on basics of blood and bone marrow, aplastic anemia, myelodysplastic syndromes (MDS), types of MDS, paroxysmal nocturnal hemoglobinuria (PNH) and clinical trials. Because some interactive modules have over 100 slides, you can go at your own pace, and just cover one section at a time, if you prefer.

Interactive Module Sample Screens
You will see slides with text and graphics with an audible spoken narration of the text shown on each slide. You can repeat a slide, skip ahead to a new section of the interactive module or even return to an earlier slide.

Some screens have callouts and also explain medical terms and concepts.

Interactive modules also help you learn by quizzing you on the content you have just covered. Questions are asked at certain intervals throughout the program and provide immediate feedback on your response, reinforcing the learning process. Interactive modules are a great way for anyone to learn about bone marrow failure diseases.

The Other Formats
Webinars
Webinar is short for “web seminar.” Webinars allow many participants to see and hear a live presentation as it happens, typically with a presenter, a moderator, and an online audience. Viewers hear the voice of the presenter and moderator and see the slide presentation. At the end of the presentation, members of the audience can ask questions which are typed in a Q&A chat window that is opened on the viewer’s computer monitor.

Webcasts
Webcasts are pre-recorded presentations that were given in front of an audience at an AAMDSIF live event. Here, viewers see a video of the presenter along with the accompanying slide presentation and the follow up questions from the audience.

Interviews with the Experts
These are pre-recorded video events in which an expert in a particular subject area is interviewed by a person who is off-camera. A list of the questions being asked is also visible on the viewer’s computer monitor. Unlike webinars and webcasts, these interviews are not accompanied by a slide presentation.

Donate a Gift of Hope to AAMDSIF

You can’t do it alone...and neither can we!

2012 Patient Education & Support Campaign

“Always stay positive in your battle against bone marrow failure diseases. Please support AA&MDSIF to help others.”

—Kirby Stone, MDS patient and AAMDSIF Volunteer

Give by mail, online or by phone at www.AAMDS.org/DonateNow, or phone (301) 279-7202 x105.
10 Questions to Ask When Looking Online for Health Information

After being diagnosed with aplastic anemia, MDS or PNH, most patients and families members will begin their search online for more information about their disease and treatment options. So, how do you evaluate health information on the Internet? Below are some tips on how you can decide if what you are seeing is credible and accurate:

1. **Who sponsors or funds the Web site?** This information may be easy to see or it may be in very small print at the bottom of the Web page. You may also have to click through links such as “about us” or “disclosures”.

2. **Is it obvious how to reach the Web site sponsor?** Trustworthy Web sites will have contact information, often including a toll-free telephone number. The site’s home page should list an e-mail address, phone number, or a mailing address where the sponsor and the authors of the information can be reached.

3. **Who wrote the information?** Authors and contributors should be identified including their affiliation and any financial interest in the information. Personal stories may be helpful, but medical advice offered in a case history should be considered with a healthy dose of skepticism. There is a big difference between a Web site developed by a person with a financial interest in a topic or product versus a Web site developed using strong scientific evidence.

4. **Who reviews the information?** Click on the “about us” page to see if there is an editorial board who reviews the information before putting it online. Are the editorial board members experts in the subject you are researching? For example, an advisory board made up of attorneys and accountants is not medically authoritative. Reliable Web sites will tell you where the health information came from and how it has been reviewed.

5. **When was the information written?** New research findings can make a difference in making medically smart choices. So, it’s important to find out when the information you are reading was written. Look carefully on the home page to find out when the Web site was last updated. The date is often found at the bottom of the home page. Remember: older information isn’t necessarily useless. Many Web sites provide older articles so readers can get a historical view of the information. Treatment information changes, so be sure to check what you are reading with your health care team.

6. **Does the site display the HONcode?** The Health on the Net Foundation Code of Conduct (HONcode) for medical and health Web sites addresses one of Internet’s main healthcare issues: the reliability and credibility of information. The HON foundation certification focuses only on human health online content.

7. **Is your privacy protected?** You want to be as certain as possible that your information is not shared with other lists or companies. Take time to identify and read the Web site’s policy—if the Web site includes something like, “We share information with companies that can provide you with products,” that’s a sign your information isn’t private. Never give out your Social Security number.

8. **Are you asked for personal information?** Be sure to find out how the information is being used by contacting the Web site sponsor by phone, mail, or the “contact us” feature on the Web site. Be careful when purchasing items on the Internet. Web sites without security may not protect your credit card or bank account information. Look for information that indicates that a Web site has a “secure server” before purchasing anything online.

9. **Are claims too good to be true?** Be wary of claims that any remedy will cure a lot of different illnesses. Be skeptical of sensational writing or claims of dramatic cures. Make sure you can find other reputable Web sites with the same information. Don’t be fooled by a long list of links. Don’t assume that a linked Web site has the same quality and accuracy. Information that sounds unbelievable probably is unbelievable.

10. **Do you want another opinion?** If you find disease or treatment information online and you’d like to know if that information or treatment option is an option for you, print out the information and take it with you to your next doctor’s appointment. Talk with your doctor before purchasing any quick cure remedies. You could actually be hindering your treatment by taking medications or herbal therapies not prescribed by your doctor.

In summary, while there’s a lot of useful, valuable and helpful information found online, there is also unreliable, unverified, and outright fraudulent information that you can easily come across. Knowing how to assess and evaluate what you find is a good place to start.
Debono Family Celebrates Son’s Legacy Through Scholarship Fund

“We are very happy to support AAMDSIF and the help that they, in turn, give to patients and families.”
--Sally and Manuel Debono

In 1984, when Matthew Debono was stricken with severe aplastic anemia, he and his family were focused on his life as a college student. Matthew was a cross-country runner who handled his disease, according to his parents, Sally and Manny, “with the same determination that he used to finish his races. He was brave and cheerful and often used humor to help him cope with his situation. At times, he would even ride his bike, IV pole attached, through the halls of the hospital.”

Diagnosed early in his freshman year at Wabash College in Indiana, Matthew always regretted not being able to receive a college education. So, he and his brother conceived of a scholarship to help students who were similarly challenged by bone marrow failure disease.

Matthew died of complications of his disease 18 months after diagnosis. The family immediately established the Matthew Debono Scholarship Fund and over 25 years, 35 candidates, eligible by virtue of having received a bone marrow transplant, have received the award. In thinking about how to expand Matt’s legacy, in 2011, the Debono family decided to re-establish their scholarship fund with AA&MDSIF so that it could make an even larger impact.

More than 200 adults and children celebrated the lives of the scholarship winners and lovingly remembered Matt at a picnic on August 6, 2011. It was an opportunity to thank donors for their generosity which enabled raising more than $70,000 and a way to mark Matthew’s 25-year legacy.

AA&MDSIF staff provided support and guidance and a free online tool where the Debonos could share their goals and ask family and friends to make secure online donations. They hope to build on their effort and will continue to preserve Matt’s legacy.

They told their family and friends that “remembering Matthew in this way is helping deserving students who suffer from the same deadly diseases that took Matt’s life.”

Attention College-Bound Students:
The AA&MDSIF Matthew Debono Memorial Scholarship Fund will award up to $15,000 in scholarships ranging from $500-$2,000 to undergraduate, graduate or vocational college students (under age 35) who are bone marrow failure disease survivors.

Promoted through the AAMDS.org Web site and elnider, applications were accepted through April 1st, 2012. Award winners will be announced in late May. Look for the application and guidelines for the 2013-2014 scholarship program in early 2013.

Create a Legacy with a Named Philanthropic Fund at AA&MDSIF

Whether you make the donation yourself, or you and your family decide to fundraise until you reach your goal, creating a named fund can make a major impact on AA&MDSIF research programs, patient education and support services, awareness and advocacy activities.

Learn more about the AA&MDSIF Major Gifts Opportunity List and ways to donate at www.AAMDS.org/Donate or contact our Development Director, Sandra Walter-Steinberg at walter@aamds.org or (301) 279-7202 x104.
Warning Signs

In September 1989, our son Bill was 15 when we started getting warning signals something wasn’t right with his health. At a routine checkup, our dentist commented his immune system must be low because of some mouth sores Bill had. Next, one of his high school teachers contacted us and says he is late for her class every morning. We questioned Bill about it, and he replied that his class was on the second floor and climbing the stairs just totally sapped his energy.

The final indicator was when we went hunting together and he just could not keep up with us old guys. I asked him if he was okay. He always said he felt good, but he didn’t look that way. He was pale and tired easily, so we took him to see our doctor. The doctors ran some tests, finding that his hemoglobin was less than three, and platelets and white cells were near zero. He was then given two units of blood and platelets. A bone aspiration test was performed and showed that he had aplastic anemia. We were then transferred to the University of Michigan Motts Children’s Hospital for further treatment.

Treatment and Transplant

A bone marrow transplant was the preferred method of treatment, but Bill’s sister was not a match. Drug therapy was then decided to be the best treatment, using cyclosporine and anti-thymocyte globulin (ATG) along with prednisone. Bill responded well, and his counts improved to about normal for about three years, but then his counts began dropping again. A second round of ATG, cyclosporine and prednisone was given, and his counts improved, but unfortunately, his counts would fall again after about a year and a half. A third and final round of ATG was given but only stabilized his counts for about 12 months, and at that time a search for a bone marrow match was conducted.

To our relief, in 1995, two people in Germany were found to be an exact match. Bill was prepped for the transplant with radiation and chemotherapy. Low doses of radiation were to be used to suppress Bill’s immune system. Mott’s Children Hospital had never performed a Matched Unrelated Donor (MUD) transplant before, so Bill was their first. Bill’s new bone marrow was flown in from Germany and administered through his IV right away.

Good News

As the bone marrow flowed through his IV, it gave us the feeling as if we were watching his birth all over again. After about 21 days in the hospital, Bill was discharged. To prevent his new bone marrow from being rejected, cyclosporine was prescribed and taken for about a year. Bill has been healthy and medication free ever since. The doctors had told us that he would probably be sterile, unable to have any children, but that turned out to be not the case. Bill and his wife have two beautiful healthy children, a girl and boy, ages 3 and 5.

A Donor’s Gift

Bill’s donor, Michael, was a college student at the time in Germany, studying to be a marine biologist. He took time out during his final exams to donate his bone marrow; he now has his doctorate degree and is a well known scientist for his studies of dolphins. We finally got to meet Michael in North Carolina where he was attending a conference and we continue to stay in touch. Michael now lives in Switzerland, is married and now has two children of his own. Bill named his son Michael to honor him for saving his life and making it possible for him to have a family of his own and for us to cherish.
THE AA&MDSIF MARKETPLACE is open for business!

Show your AA&MDSIF spirit – and help spread awareness – with our practical and stylish Marketplace items!

AA&MDSIF items are now available through our online Marketplace including our popular Bravery bracelets. By purchasing Marketplace items, your support helps raise awareness and also helps fund the patient education, research, awareness and advocacy programs of AA&MDSIF.

**Men's and Women's Red Polo Shirts ($30 per shirt)**
Classic short sleeve shirt with AA&MDSIF in white stitching and the Answers, Support and Hope logo in black stitching.
Sizes:  
- Men’s Sizes: M-XXL
- Women’s Sizes: S-XL

**Canvas Tote bags ($30 per bag)**
Large boat-tote in natural with red straps and embroidered logo.

**Canvas Hats ($20 per hat)**
Six paneled hat with adjustable strap in natural with Answers, Support & Hope logo embroidered on the front and www.AAMDS.org stitched on the back.

Shipping for the above items:
- (shirts, totes, hats) 1 item: $6.00
- 2-3 items: $12.00
- 4 or more items: $15.00

**Bravery bracelets ($10 for 10 bracelets/1 pack)**
Red and white silicone bracelets, symbolic of red and white blood cells.

Free shipping for up to 50 bracelets/5 packs.
For orders of 6 packs or more, call AA&MDSIF (800) 747-2820.

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**Volunteer Vibe**

It all began in January 2010, when Robert called AA&MDSIF because he wanted to organize an event in honor of his wife Kelly, an aplastic anemia survivor for over 20 years! In his own words, he wanted to hold this event because:

“This disease could take my wife at any time without notice. I can’t sit around and let that happen and I need your help to change this... not just for Kelly... but for everyone fighting this disease.”

By organizing the Jackson County Brevet bike event, Robert knew he could make a difference by spreading awareness about aplastic anemia and raising money in support of AA&MDSIF’s programs, services and research. As we worked with him to plan his first event, he said he wasn’t an expert in event planning, but he was a cyclist and a certified cycling coach, who owned his own cycling training business, so he knew the cycling community.

Robert decided to organize the 1st Annual Jackson County Brevet in the northwestern area of Atlanta, not only because it was an area he knew well, but because there weren’t any major cycling events in this part of Atlanta. He believed he could get cyclists interested in participating. He planned the event, along with a great team of friends and volunteers, and expected about 100 cyclists to participate and about $5,000 to be raised.

Because of Robert’s non-stop, hard work in organizing and promoting this event, there were just over 600 riders with $25,000 raised at the 1st Annual Jackson County Brevet! It was an incredible day for the participants, volunteers, patients and their families who attended.

So Robert decided that 2011 would be even bigger and better! Because he already had the framework in place from planning the first event, he knew he could almost double the amount of participants and money. And he did! There were 1,000 registered riders and over $35,000 raised. Robert distributed 20,000 flyers over the Atlanta area to let people know about this event.

This incredible event is the largest volunteer organized event held for AA&MDSIF. It has surpassed everyone’s wildest expectations! And it all started with one volunteer with one idea.

The 3rd Annual Jackson County Brevet will be held on Saturday, June 16, 2012. You can learn more about this event at www.jacksonbrevet.com.
Get Involved….Give Back! Help Raise Awareness and Funds for AA&MDSIF!

Fundraising events come in all shapes, sizes, and forms with a variety of themes. It is best to plan an event that interests you. What’s the key to planning a successful fundraising and awareness event? Do what YOU know!

Organize a local Hope, Steps & A Cure Walk!
With the help of AA&MDSIF, you can plan a fun and festive walk in your own community to raise awareness about bone marrow failure disease while raising much needed funds for AA&MDSIF programs and services to help patients and families. In 2011, AA&MDSIF volunteers organized walks in San Antonio, TX; Los Angeles, CA; Detroit, MI; and Winter, WI. And many more walks are already being planned in 2012, taking place in San Antonio, TX, Los Angeles, CA, and Milwaukee and Oshkosh, WI! Learn more at www.AAAMDS.org/walk.

Host an AA&MDSIF House Party!
Invite family, friends and neighbors to your home for a brunch, dessert party, happy hour or afternoon tea and tell your/your loved one’s story about fighting a bone marrow failure disease. Have fun raising awareness and funds. This is a fun, yet simple way to get involved.

Plan a fun event!
Plan an event around an activity that you know well. In 2011, AA&MDSIF volunteers planned a wide variety of exciting and successful activities, including: TS Gates Memorial Run/Walk, Jackson Brevet bike event, Torry Yahn Barbeque and Auction, Judy Joyce Memorial Golf Tournament, Michael Farreny Volleyball Tournament, Pin Down MDS Wrestling Tournament, Classical Guitar Concert, Breakfast for Aplastic Anemia, Halloween Benefit Bash, Catherine’s Craft Fair, and more! The key to their successes? They did what they knew!

So how do you want to get involved and give back? You can learn more at www.AAMDS.org/Get-involved.

Help Spread Awareness — It’s Vital — and it’s Easy!

Spreading awareness about bone marrow failure disease is one of the most significant ways people can help AA&MDSIF. Because these diseases are rare, it is important to educate others about what aplastic anemia, MDS, PNH, and other bone marrow failure diseases are. It’s also important to draw attention to our mission and message so that others affected with a bone marrow failure disease—who may not yet know about AA&MDSIF—can connect to us and receive the information and support they need.

There are many easy and effective ways to help AA&MDSIF spread awareness in your own community.

1. Share your story
Share your or your loved one’s experiences with a bone marrow failure disease. Let others know how you have been affected and how you have coped with your disease. Share your story by organizing a lunchtime talk, writing a blog, contacting your local paper, or using social media like Facebook.

2. Spread awareness in your local and global community
Locally, you can hand out AA&MDSIF brochures to health professionals and medical institutions, distribute our awareness bracelets that you can purchase on our Web site, or advocate to your U.S. representatives.

Globally, you can let others know about bone marrow failure disease and AA&MDSIF through social media, blogs, email, and postings on community calendars. Forward the monthly eInsider to family, friends, colleagues, and your healthcare professional. They will learn more about these diseases, help them understand what you are facing, and this might even help someone else they know who has yet to be diagnosed.

3. Set up a simple awareness event
Organize a bone marrow/blood drive and help other patients who are in dire need of a bone marrow transplant. By distributing AA&MDSIF brochures, you can educate others in your community about bone marrow failure disease.
Awareness

Patients and Families Connect through AA&MDSIF Communities of Hope

Communities of Hope are volunteer-led local groups, working together with AA&MDSIF staff, connecting patients and families with each other. AA&MDSIF is building Communities of Hope in towns across the country and seeks volunteer support for this patient-oriented initiative.

Communities of Hope are being built:

- To connect patients and families with each other to provide a local resource for peer support, education, and information exchange
- To connect patients and families with us, as a means for carrying out AA&MDSIF’s mission
- To engage volunteers and local professional resources for generating awareness among the patient and health professionals community, as well as the greater community
- To create advocacy and support for AA&MDSIF
- To tap into geographic location to raise support for AA&MDSIF and to help carry out AA&MDSIF’s mission

Each community will look slightly different from another. It’s up to the members to decide how they want their group to function. Each community will determine what activities they’d like to participate in – all with the help and support of AA&MDSIF!

Groups for mutual support

Some communities may function solely as patient support groups. These groups will meet on a regular basis so that members can talk about the issues important to them, discuss their experiences, and share stories as they cope with bone marrow failure disease.

Other communities may function as an enhanced support group. They’ll meet in a support group capacity, but they’ll also invite local health care professionals to talk to them about topics of importance to the group.

Awareness and fundraising-oriented groups

Still other communities may join together to organize and participate in a specific fundraising and awareness event in their community. Members in these communities may plan a Hope, Steps & A Cure Walk, a golf tournament, restaurant fundraiser, bone marrow drive, or outreach program to health care professionals. In participating in these events, they will spread awareness about bone marrow failure disease in their own community while also raising much needed funds in support of AA&MDSIF programs and services.

Advocacy and action-oriented groups

And finally, other communities may work together in advocacy efforts. Members in this group will advocate to their local representatives and senators to carry out AA&MDSIF’s advocacy work. Whether there is pending legislation or simply a need to educate about bone marrow failure diseases, advocacy to elected representatives remains important.

Make your Community of Hope meet the needs of its members

In building a Community of Hope, there’s no one size fits all approach. Shape your own community to the interests of your members. Decide what activities best suit your group and determine what your group will do and how and when you’ll do it. Best of all, you’ll be connecting with other patients and families, like yourself, as you cope with and manage your or your loved one’s bone marrow failure disease.

AA&MDSIF is with you every step of the way - so start now!

AA&MDSIF will be there to support your group! We’ll help you started and organized, as you decide what shape your community will take. We’ll help connect you with other patients and families to provide you with the support, resources, advice, and information you will need to have a successful, growing Community of Hope.

Join or create a Community of Hope

Communities of Hope have already formed in Asheville/Hendersonville, NC; Baltimore, MD; Central NJ; Cincinnati, OH; Lima, OH; Middletown, CT; Phoenix, AZ; Raleigh/Durham, NC; Seattle, WA. Communities are in the process of forming in Boston, MA; Chicago, IL; Dallas, TX; Detroit, MI; Minneapolis and St. Paul, MN; Philadelphia and Pittsburgh, PA; and Tampa, FL. Do you live in one of these areas and would like to join? Or would you like to start a Community of Hope in your town? Contact Community Development Manager Martha Crews at crews@aamds.org or (301) 279-7202 x103 to get connected to an existing Community of Hope or to start a new one in your area.
Free Phone Support for Personal Attention

Do you need to speak with someone directly? Please contact Leigh Clark, our Patient Educator, at (800) 747-2820, option 1, or by email at info@aamds.org. Leigh communicates with people all over the world, answering a wide range of questions, including information on treatment options, clinical trials, financial resources, and more.

PEER SUPPORT NETWORK

Let this AA&MDSIF resource help you!

The Peer Support Network is a national network of trained volunteers, including patients, caregivers, and family members, who offer information, personal experience, coping strategies, problem solving skills and informational resources to people just like themselves. Speaking with a Peer Support Network volunteer is a great way to gather information and receive emotional support from someone whose life has also been affected by bone marrow failure disease.

Call now!

To connect with a Peer Support Network volunteer, call (800) 747-2820, option 1, and speak with our patient educator, Leigh Clark, who will match you with one of our volunteers. You can also email her at info@aamds.org.

Online...

AA&MDSIF Webinars and Webcasts

AA&MDSIF presents webinars by some of the nation’s top experts in bone marrow failure diseases and on living well with these diseases. For more information or to register for upcoming webinars, visit our Online Learning Center at www.AAMDS.org/Learn.

Join the AA&MDSIF Facebook Community at www.facebook.com/aamds

Over 2,500 people communicate with each other and with AA&MDSIF through our active and informative Facebook page.

Follow Us on Twitter at www.twitter.com/aamdsif.

Meet Up On

Marrowforums hosts free online discussions about bone marrow failure disease. Ask questions. Exchange information and support. Marrowforums is run by patients and caregivers just like you! Register now at www.Marrowforums.org.

In Print...

Free Patient Guides, Fact Sheets, and Additional Resources

Request a patient packet from AA&MDSIF, containing a patient guide on aplastic anemia, MDS or PNH, and fact sheets with critical information for all bone marrow failure disease patients, families, and caregivers.

Brochures and Fact Sheets

- AA&MDSIF Advocacy
- AA&MDSIF Brochure
- AA&MDSIF Social Media
- Bone Marrow and Stem Cell Transplantation
- Clinical Trials
- Communities of Hope
- Financial Resources
- How to Evaluate Health Information on the Internet
- Iron Overload
- Marrowforums Hosted Online Discussions
- Online Learning Center

Patient Guides

- Your Guide to Understanding Aplastic Anemia
- Your Guide to Understanding MDS
- Your Guide to Understanding PNH
- Living Well With Bone Marrow Failure Disease
- Standing Up for Your Health
- What to Expect From Treatment: A Guide to Understanding FDA-Approved Drug Therapies for Myelodysplastic Syndromes (MDS)

To order a patient packet, call (301) 279-7202, x116, or order online at www.AAMDS.org/Info.
Address or email change?  
Want to be taken off our mailing list? 
Return this page with your comments, email update@aamds.org or call (301) 279-7202 x105.

What are Bone Marrow Failure Diseases? 
Aplastic anemia, myelodysplastic syndromes (MDS), and paroxysmal nocturnal hemoglobinuria (PNH) are rare bone marrow failure diseases.

Aplastic Anemia

Aplastic anemia occurs when the bone marrow stops making enough red blood cells, white blood cells or platelets for the body. Any blood cells the bone marrow makes are normal, but there are not enough of them. Aplastic anemia can be moderate, severe, or very severe.

Myelodysplastic Syndromes (MDS)

MDS is a group of disorders where the bone marrow does not make enough healthy blood cells. All MDS types have a low blood cell count for at least one blood cell type (red blood cells, white blood cells, or platelets), and the bone marrow and blood contain some blood cells that are abnormal in shape, size, or function.

Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is a blood disease that causes red blood cells to break apart, a process called hemolysis. The broken cells are then released in your urine.

Approximately 15,000 to 18,000 people in the United States are diagnosed with one of these diseases every year. AA&MDSIF provides answers, support and hope for patients, families, and caregivers whose lives are impacted by bone marrow failure diseases.

For complete descriptions, please visit www.AAMDS.org/Diseases