AA&MDSIF is pleased to announce two resources for patients are now available in French, German, Italian, Portuguese, and Spanish.

Patient Guides

Because patients are looking for answers to the many questions that arise when they are diagnosed with a rare bone marrow failure disease, AA&MDSIF provides expert, reviewed medical information in our comprehensive patient guides.

• Your Guide to Understanding Aplastic Anemia
• Your Guide to Understanding MDS
• Your Guide to Understanding PNH

Video interviews with MDS experts discussing:

1. What is MDS and how is MDS diagnosed?
2. What is the difference between supportive care and active treatment for MDS?
3. How are decisions made about the best approach to treat a patient?
4. What new treatments are on the horizon for MDS patients?

Log on to www.AAMDS.org/Global to view the videos and read or print the patient guides in French, German, Italian, Portuguese, and Spanish.

New Directions in Research Advanced at 2014 Scientific Symposium

The Fourth AA&MDSIF International Bone Marrow Failure Disease Scientific Symposium, “Building a Collaborative Research Community That Saves Lives,” was held on March 27 and 28, 2014 in Rockville, Maryland. The symposium gathered over 160 participants to hear more than 35 of the world’s leading researchers on aplastic anemia, myelodysplastic syndromes (MDS), and paroxysmal nocturnal hemoglobinuria (PNH) share the latest findings, discuss current areas of controversy, and propose specific recommendations for the highest priority directions for basic and clinical research.

Once considered distinct, aplastic anemia, MDS, and PNH are now believed to be linked by similar pathophysiologies. Exploration of current research issues will greatly benefit from increased collaboration between basic and clinical scientists and between scientists studying the individual diseases.

► Continued on page 5
In 2014, we have been focused on three R’s—Research, Reach, and Resources. Feedback from you, an analysis of our current programs and services, and our continuous desire to serve you better keeps us patient-centered and patient-focused.

Research

Patients and family members have told us that they closely follow research developments weekly (20%), monthly (26%), and quarterly (24%) – that’s an overwhelming 70% who are interested in learning the latest research findings that may impact their care and treatment. We have expanded our efforts to share even more research information with patients, family members, caregivers, and health professionals through our conferences, webinars, print and electronic publications, and website. We have expanded our research summaries, a twice yearly publication which summarizes the latest findings from the major scientific meetings. We have also added a monthly electronic Research Connection newsletter to focus on new developments in research and new clinical trials.

Reach

Every year, we serve more patients, families, and healthcare professionals than ever before. Our database is just about at 50,000, but we know we have a long way to go. We will expand our reach to deliver our services to specific audiences – adolescents in transition to young adults; a greater number of PNH patients; and in particular, to the caregivers who are so often overlooked.

Beyond our conferences and webinars, we will be increasing the number of live webcasts from our conferences and also as stand-alone sessions. Increased use of social media has already shown us how we can reach more people, and we are focused on those efforts along with continued improvements to our website. A significant challenge is to reach those who are not yet connected to us. Increasing our outreach efforts to community-based hematologists and oncologists through professional education programs, e-communications, and electronic tools is high on our list. The patients we are trying to reach are in those doctors’ offices, so we need to be there, too.

Our new global awareness efforts and leadership of the new MDS Alliance will open up new avenues for reaching many more patients and will enable us to work collaboratively with our sister organizations world-wide.

Resources

Expanding our donor base and levels of support is always a challenge. We know there are always more people to help and more services to provide, and the support and contributions we receive from so many patients, families, friends and others make it possible to offer ALL of our services at no cost to you. Without that support, we could never provide the answers, support and hope to the thousands of people who seek our help. So to fund and provide more Research, and to Reach more people, we need to continue to attract more Resources. Now halfway into the year, we haven’t just learned the three R’s, we’re living them.

Stay well,

John M. Huber
Executive Director

MISSION

The Aplastic Anemia & MDS International Foundation (AA&MDSIF) is an independent nonprofit organization whose mission is to support patients, families, and caregivers coping with aplastic anemia, myelodysplastic syndromes (MDS), paroxysmal nocturnal hemoglobinuria (PNH), and related bone marrow failure diseases.

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About Us

Fighting Bone Marrow Failure Diseases Through Patient Support and Research Since 1983
Meet the 2014 AA&MDSIF Grant Recipients

For 25 years, AA&MDSIF has provided research grants totaling over $4 million to an international group of more than 67 researchers to help advance the understanding and treatment of aplastic anemia, myelodysplastic syndromes (MDS), and paroxysmal nocturnal hemoglobinuria (PNH). These two-year grants have helped bring forth new insights into the causes and therapeutic approaches for these diseases. These profiles present the newest group of grantees and a summary their grant-funded research projects.

Daria Babushok, MD, PhD
Fellow in Hematology/Oncology
Hospital of the University of Pennsylvania

Funded by: Torry Yahn, Sam Jordan and Stephen and Joanna Janowiak Research Funds

Clonal hematopoiesis and autonomous lymphocyte expansion in acquired aplastic anemia

Acquired aplastic anemia (aAA) is a devastating blood disorder, affecting children and adults, caused by immune attack on the bone marrow. Factors that determine recovery, relapse, and transformation to myelodysplasia in aAA remain poorly defined, and there is a dearth of studies in pediatric patients. Emerging data suggest that mutations in the bone marrow and in the pathogenic immune cells contribute to disease evolution and relapse. To study this, I will employ cutting-edge genomic techniques to characterize pathogenic mutations in aAA. These studies will improve treatment of patients with pediatric aAA by allowing early detection of clonal evolution and disease progression.

Luis Batista, PhD
Assistant Professor of Medicine
Hematology, Assistant Professor of Developmental Biology
Washington University in St. Louis

Funded by: Bradley Richard Research Fund and Research is Hope Fund

Improving the self-renewal capability of stem cells derived from bone marrow failure patients

Bone marrow failure is a serious condition that occurs when the bone marrow stops making enough healthy blood cells. A risk for bone marrow failure is genetic instability, including exacerbated shortening of telomeres (repetitive DNA sequences that cap chromosome ends). Using different genetic and biochemical approaches, this proposal will use cells derived from bone marrow failure patients that have telomere attrition as a platform for the development of clinical therapies against this disease. These experiments will increase our knowledge on stem cell function and regulation in bone marrow failure syndromes.

Rosannah Cameron, PhD
Research Fellow
Department of Developmental & Molecular Biology
Albert Einstein College of Medicine

Funded by: Lindsay Minelli Research and the Research is Hope Funds

The role of spliceosome in regulating DNA methylation in myelodysplastic syndrome

We do not yet fully understand the process of disease progression in myelodysplastic syndrome (MDS). We

Continued on page 4
know certain genes are involved because mutations in those genes have been found in MDS patients. We need to understand which genes are important early in the disease and which ones act later. Our goal is to identify the genes that act early in MDS, so that ultimately, our research will lead to new treatment options that are more specific and effective.

Younma Kfoury, PhD
Research Fellow
Massachusetts General Hospital,
Center for Regenerative Medicine
Funded by: Lisa Lancaster Research Fund

Phenotypic and molecular characterization of osteolineage cells in patients with myelodysplastic syndrome

Myelodysplastic syndromes (MDS) are a group of diverse and incurable pre-leukemic disorders. Even though a deregulated bone marrow microenvironment is thought to participate in the disease, its role remains elusive. Since the microenvironment is better understood in mice, we have identified a subset of cells in the human bone marrow that is equivalent to those cells in mice which, when perturbed, lead to MDS. We propose to characterize these cells in patient samples at the cellular and molecular level. This will provide a better understanding of the abnormal signaling pathways involved in MDS, and eventually identify therapeutic approaches.

Patrizia Ricci, PhD
Department of Clinical Medicine and Surgery
University of Naples, Italy
Funded by: PNH Research and Support Foundation

Small peptide C3-inhibitors for the treatment of paroxysmal nocturnal hemoglobinuria: moving towards the bedside

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare hematological disease characterized by spontaneous destruction of red blood cells (intravascular hemolysis), poor functioning of bone marrow, and thrombosis. Recently, the availability of the complement inhibitor eculizumab has dramatically improved the treatment of PNH. Nevertheless, about half of the patients treated with eculizumab shows a persistence of clinical signs of the disease. We have recently described the reasons underlying the limited efficacy of current anti-complement treatment for PNH. With this proposal we aim to complete the pre-clinical development of a novel complement inhibitor which targets early events in complement activation, the component-3 of the complement cascade.

Chao-Yie Yang, PhD
Research Assistant Professor
University of Michigan
Funded by: PNH Research and Support Foundation

Discovery of small molecule complement inhibitors as the treatment for PNH

Uncontrolled complement activation caused by a gene (PIG-A) mutation in hematopoietic stem cells has been characterized in PNH. Patients suffer from hemolysis, thrombosis and bone marrow failure. Current FDA-approved only treatment for PNH is eculizumab which is expensive, unable to eradicate PNH clone, not orally available, requires clinic-bound infusions via iv access and lifelong therapy. In this proposed work, we will discover and make rational designs to small molecules guided by protein structures to develop complement inhibitors. The promising small molecule inhibitors identified from this work will be evaluated and used to develop orally-available therapeutics to treat PNH patients.

MDS Clinical Research Consortium Members Present Research

Several researchers at member institutions of the MDS Clinical Research Consortium presented their research at the December 2013 American Society of Hematology Annual Meeting. Among them were:

David Steensma, MD
Dana Farber Cancer Institute

Disparity in Perceptions of Disease Characteristics, Treatment Effectiveness, and Factors Influencing Treatment Adherence Between Physicians and Patients With Myelodysplastic Syndromes

Eric Padron, MD
H. Lee Moffitt Cancer Center and Research Institute

Outcome Of Patients With Low and Intermediate-1 Risk Myelodysplastic Syndrome (MDS) After Hypomethylating Agent (HMA) Failure.

The MDS Clinical Research Consortium allows the clinical researchers and fellows who are funded through the Edward P. Evans Foundation, to collaborate on clinical trial research among the six institutions.
The exchange of ideas and opinions on current research that occurred at the symposium is resulting in collaboration among investigators toward new understanding of the diseases, networks to further individual research, and new approaches on how to transform findings into treatments.

Key Findings

Several important clinical and scientific advances made recently in bone marrow failure disease research were identified at the symposium. These included:

- Better understanding of the molecular biology of bone marrow failure diseases has resulted in clinical benefit, with many recent therapeutic advances based on genetic research
- The genomic landscape of MDS is a key area of advancement, as 90% of MDS patients have at least one genetic mutation
- Studies of eltrombopag (Promacta®) show promising results for aplastic anemia
- Research advances understanding of immunity and immunology in PNH

These findings and other new insights resulting from the symposium will have an impact on both research and improved patient care for years to come.

Interviews were recorded at the symposium with the session co-chairs who explain the highlights of the presentations for patients. These interviews may be viewed at www.AAMDS.org/SymposiumVideos.

2013 Leadership Awards Announced at Scientific Symposium

AA&MDSIF announced its Leadership in Philanthropy, Science and Service Awards at the 2014 International Bone Marrow Failure Scientific Symposium on March 27.

The Leadership in Philanthropy Award is given for continued and consistent generous financial support of AA&MDSIF programs and services. The Edward P. Evans Foundation was selected for the 2013 Leadership in Philanthropy award in recognition for its support of the AA&MDSIF MDS Clinical Research Consortium.

Mikkael A. Sekeres, MD, MS was selected for the Leadership in Science award. Recipients are chosen for their contributions to the fields of bone marrow failure disease treatment and/or research. Nominees are considered for their impact on patients and families affected by aplastic anemia, MDS and PNH, and/or their contributions through research to increasing knowledge and understanding about these diseases. A frequent speaker and presenter for AA&MDSIF patient and family conferences and webinars, and webcasts and videos for both patients and health professionals, Dr. Sekeres is co-chair of the AA&MDSIF Medical Advisory Board, and also co-chair of the MDS Clinical Research Consortium.

Neil Horikoshi was selected for the Leadership in Service award. Recipients of this award are chosen for their demonstrated commitment of time, energy and ideas that raise awareness for AA&MDSIF and bone marrow failure diseases.

He is a past chairman of the AA&MDSIF Board of Directors, and led its transformation to a strategic and policy making governance Board. He is an Advisory Council member for both the Asian American Justice Center (AAJC) and the Asian Pacific American Institute for Congressional Studies (APAICS). He also serves as an Advisory Council member for the Bill & Melinda Gates Foundation’s Gates Millennium Scholars Program.

“We are deeply honored by the immeasurable contributions of the Edward P. Evans Foundation, Dr. Mikkael Sekeres and Neil Horikoshi toward the success of AA&MDSIF, and are pleased to honor them with these special awards,” said AA&MDSIF Executive Director, John Huber.
AA&MDSIF – Your Resource of Choice

AA&MDSIF’s staff and medical advisory board members know that a diagnosis of a bone marrow failure disease is a scary moment for patients and their loved ones. We help you understand your diagnosis and treatment options, and help you determine the best way to manage your life with aplastic anemia, MDS, or PNH.

We also know that people learn in different ways, and that the volume of information available can be overwhelming - like trying to get a sip of water from an open fire hydrant! So we are here to help you in many ways and in many formats. Here are a few of our best resources.

Patient Guides and Fact Sheets

Patients often look for answers to the many questions that arise when they are diagnosed with rare bone marrow failure diseases. AA&MDSIF provides expert reviewed medical information in our patient guides and other educational materials. Call (800) 747-2820 to request your free copy!

Patient and Family Conferences

We are meeting you where you are in 2014 by offering three more free regional conferences on aplastic anemia, MDS, and PNH. Each program will provide you with answers from leading medical experts and the chance to connect with as many as 150 other patients and their caregivers. Join us—learn about your bone marrow failure disease and know that you are not alone. Visit www.AAMDS.org/Conferences or call us (800) 747-2820 to register for a conference.

Online Learning Center

You can find upcoming and previously recorded educational sessions, interviews, and interactive learning modules at www.AAMDS.org/learn. Sign up for live webcasts and webinars by disease or check to see if your doctor is one of our medical experts.

New Bone Marrow Transplant Tutorial Available in English and Spanish!

Bone marrow or stem cell transplant transplantation is the only cure for some bone marrow failure diseases. The Aplastic Anemia & MDS International Foundation recently received a grant from the Mia Hamm Foundation* to support the creation of our new interactive learning module focused on bone marrow transplantation.

The decision to undergo a bone marrow transplant is challenging because of the risks involved before, during and after transplant. The Bone Marrow Transplant (BMT) Tutorial Module provides visual animation, questions, and feedback to enhance participant understanding of BMT as a treatment option. This new module, available in English and Spanish, is also helpful to family members so they can better understand transplantation and provide support for their loved one.

The AA&MDSIF Online Learning Center currently offers interactive modules on aplastic anemia, MDS, and PNH, as well as modules on blood and bone marrow basics, and clinical trials. Visit www.AAMDS.org/Learn and select the BMT Tutorial under Interactive Modules.

*The Mia Hamm Foundation is a non-profit, national organization dedicated to raising funds and awareness for families in need of a marrow or cord blood transplant and to the development of more opportunities for young women in sports. The Mia Hamm Foundation has generously partnered with AA&MDSIF to provide education and support services for patients and families seeking information about bone marrow transplantation through a grant to support this interactive learning module.
Follow Your Treatment with Treatment Tracking Tools

For patients undergoing treatment for aplastic anemia, MDS, and PNH, treatment can be long and improvement can be slow, with repeated treatment cycles needed before effects are felt. AA&MDSIF is pleased to offer new Treatment Tracking Tools to help patients monitor their daily symptoms and record their reaction to therapies and treatment progress. This information helps patients see even small changes in how they feel with treatment.

How it Works

Use the Symptom Snapshot section to track and record the day-to-day symptoms and side effects related to your bone marrow failure disease and treatment. The total snapshot score will help you see even small changes over time. You can also track your treatments along with your lab work results so that you can easily see how your treatments affect your overall quality of life.

You can share the information you record on the app or spreadsheets with your doctor or family. The information you record with the Tracker app or spreadsheet version can be sent only to the individuals you designate. With the smartphone app, you can even send it to yourself to keep a copy on your computer!

The Tracker app is available for smartphones and iPads. It also comes in an Excel spreadsheet version for personal computers, and if you still prefer to use paper and pencil, we have that too!

Researchers are always looking for new and better ways to treat MDS.

Clinical trials:

- Help scientists learn more about standard treatments
- Test the safety and effectiveness of new treatments
- Compare new treatments or new combinations of treatments, or compare these treatments with standard ones acting as a comparison group

Be sure to explore www.clinicaltrials.gov!

Visit www.clinicaltrials.gov, a Web site maintained by the U.S. National Library of Medicine (NLM) at the National Institutes of Health (NIH). Intended for patients, family members, healthcare professionals, and other members of the public, this fully searchable site provides easy access to information on clinical studies on a wide range of diseases and conditions.
In addition to the informative presentations that meet you where you are in your disease journey, attending an AA&MDSIF conference encourages you to interact with other patients, family members and caregivers. Conference participants can customize their experience by selecting from three breakout sessions focused on different areas of “managing your new normal”.

When you attend an AA&MDSIF conference, you will:

• Learn best practices from healthcare specialists that you can apply in your own experience.
• Inspire others with your story.
• Build a support network that can help you cope and be a more effective self-advocate for your best healthcare.
• Make lasting connections with other people who truly understand what you are going through.

Here’s what attendees are saying about their conference experience:

“I learned more today at this conference than I have anywhere else!”

“Attending this conference, learning from the experts, and most of all meeting others that truly understand what it’s like to have one of these diseases has helped me finally learn to breathe easier!”

“I have been an oncology nurse for 20+ years and have never heard such a good explanation of what MDS is. AA&MDSIF always brings speakers that the attendees can understand, and also approach if they have questions.”

“I now know this disease shouldn’t stop me from living life as best I can.”

Diagnosis, Treatment, and Managing the New Normal

Agenda

(SEE WEBSITE FOR MORE DETAILED AGENDA)

7:30 - 8:30 AM  Check-in and Breakfast
8:30 - 8:50 PM  Conference Welcome
9:00 - 10:30 PM Session A: Your Life Changing Phase of Diagnosis
10:30 - 10:45 PM Break
10:45 - 12:15 PM Session B: Your Life Changing Phase of Treatment
12:15 - 1:45 PM  Lunch and AA&MDSIF Program
2:00 - 3:00 PM  Session C: The Life Long Phase of Living with a Chronic Disease - Managing Your New Normal
3:00 - 3:15 PM  Break
3:15 - 5:00 PM  Session D: Finding Strength in Numbers - Peer Support Forums

Special thanks to Celgene Corporation, Amgen, Inc., and Novartis Oncology for providing support for this program.
Interviews with the Experts
Emotional Stress: Advice for Patients

Isabel Schuermeyer, MD
Cleveland Clinic

Isabel Schuermeyer, MD, is director of psycho-oncology at the Cleveland Clinic. She joined the staff in after completing a psychosomatic medicine fellowship at Cleveland Clinic. Her specialty interests are psycho-oncology, bioethics, psychiatric side effects of cancer treatment, and improving adherence to medical treatments in the cancer population.

Here, she speaks about techniques for coping with a bone marrow failure disease diagnosis and suggestions for how patients can remain focused and resilient while being treated.

In your work with bone marrow failure disease patients, what are the common emotional and psychological symptoms and reactions you have observed?

Some of the common emotional responses we see in the early stage of diagnosis include feeling “shocked” or betrayed – where patients think, “How could my body have let me down?” Others may feel confused, centering on “what does this illness mean?” – primarily because it’s a rare disease that is unknown to many until it happens to them. And certainly, newly diagnosed patients feel overwhelmed. However, after this initial phase, most people are able to move forward. For those patients who have significant distress during the time, we recommend psychotherapy with the goal of focusing on more healthy coping mechanisms.

As mentioned above, most people are able to move forward emotionally after the initial shock of the diagnosis, typically within two to four weeks. However, about 30% of patients progress to true depression or anxiety disorders that interfere in their daily lives. This is characterized by feeling down or depressed every day for two weeks, as opposed to what everyone has – about one bad day a week. For patients with either a depressive disorder or an anxiety disorder, more intensive treatment is warranted, often including psychiatric medicines such as antidepressants.

With regard to caregivers, they typically do very well during the initial diagnosis phase. However, they can develop emotional distress once the treatment plan is in place and the patient has been able to move forward emotionally. After that adjustment period, we want to see the caregivers going back to things that they love doing, not spending 100% of their time focusing on the patient or giving up things that they enjoy so that they can always be watching the patient.

Can there also be physical complications that arise from mental and emotional stress?

It’s important to distinguish between physical symptoms that may be from the disease itself and the emotional consequence of the disease. For example, patients not wanting to participate in some activity that they previously engaged in – such as playing golf – could be due to the fatigue that often is associated with a bone marrow failure disease. But if the reason is loss of interest – that is a warning sign that could indicate depression or an anxiety disorder. We know that emotional reactions that can happen may cause worsening physical symptoms, because patients may not adhere to treatments – for example, missing transfusions, not taking daily medicines, which can have serious effects on their illness.

If a patient is having difficulty coping, he or she may not require medical treatment. Seeing a therapist can be very beneficial. Many studies have shown that psychotherapy can help patients improve already existing coping mechanisms and develop new coming techniques. In addition, psychotherapy is an excellent treatment for depression and/or anxiety.

Medicines can also be used to treat depression or anxiety disorders. However, they should not be taken just for difficulty with adjusting to a serious illness. These medicines are taken daily and as with any medicines, they do have the risk of side effects. Both depression and anxiety are very common illnesses and having had a history of either puts a person at higher risk of developing another depressive episode or return of anxiety in the setting of a new medical diagnosis.

Many times, we recommend both a medicine along with psychotherapy for patients with depression or an anxiety disorder. Sometimes patients will opt for one treatment or the other for a variety of reasons. Some patients don’t want to do psychotherapy because it is time-intensive; however, psychotherapy has excellent outcomes, and there are no drug-drug interactions. Others will opt for a medicine alone. Regardless, if someone is having true major depression or an anxiety disorder, some type of treatment is warranted.

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**Are some coping strategies known to be more effective and more helpful than others?**

As to coping mechanisms used by patients—there are healthy ones, less healthy ones, and some that are just plain unhealthy. The obvious unhealthy ones are alcohol and drugs – these change brain chemistry further into an unhealthy state. Healthy coping mechanisms include things like humor, taking one day at a time, and having realistic optimism. One thing to note is that an earlier, popular belief that positive thinking alone improved cancer outcomes has been discredited. Patients should use what has worked for them in the past. Developing coping skills is a lifelong endeavor – everyone’s had stress. So the healthy coping mechanisms you have used before are the ones you should try now. A radically different set of coping tools apart from your normal ones probably won’t help and will likely lead to much frustration.

**How do you help patients cope with these and other emotional symptoms that arise from being diagnosed with bone marrow failure diseases?**

I want to know what patients are currently doing to manage stress, and I start by asking how they have coped with stressful situations in the past. I have them specifically identify what they already do to deal with stressful things at work, at home, at school, and other life situations.

There are many things that can help with stress reduction. Relaxation techniques are important, and if patients can’t immediately identify one they currently use, then it is time to learn. An easy relaxation technique is deep breathing. This can be learned pretty quickly and with some practice, patients can turn to in times of stress. Others include ‘journaling’ or writing down thoughts and responses and meditation.

Routine physical exercise is also an excellent way to wind down stress. Sometimes bone marrow failure disease patients who experience fatigue can’t maintain an earlier exercise regimen and need to find alternative exercise plans.

To summarize, turning to your supports, such as calling a friend, along with relaxation techniques and exercise, can be excellent ways to handle the stress of a chronic illness. Patients need to experiment and find what works best for them.

**How do support groups help?**

From my perspective, support groups and networks are exceptionally valuable. But if patients say they aren’t interested in participating or feel they will not benefit from participating in a support group, I may ask them to at least try it once or twice. Most large cities have a patient support organization where these types of groups are formed and conducted. Many will also have other types of activities, including exercise classes. So if a patient does not want a support group, I might suggest they use the services of the organization in some other beneficial way. While an exercise class at one of these organizations is not a classically defined support group, this can result in being in a supportive environment. Many of my patients will do this type of class as a segue into doing a more formal support group.

**Are there any new trends or directions in the practice of counseling patients who are having stress-induced and emotional difficulties?**

There are some excellent studies looking at cognitive behavioral therapy in the setting of severe medical illness. These studies have shown that this type of therapy is effective in the treatment of depression and anxiety disorders, along with improving coping techniques. Cognitive behavioral therapy is based on the theory that what you think influence how you feel.

**What is the most important advice you can give patients for effectively coping with their bone marrow failure disease?**

I can’t emphasize enough that what has worked in the past is what will work for you now. When someone is facing a serious medical illness, trying to completely change your coping mechanisms is a mistake and will result in much frustration. Strengthen your healthiest coping techniques and eliminate any unhealthy practices. Try to recognize if you are slipping into depression or anxiety disorders, and seek help if you think things are getting worse.

If you are having a depressed mood or a decrease in your interests for over two weeks, you should get help now. Depression and anxiety disorders can affect your quality of life and your ability to adhere to a treatment schedule, resulting in worsening of the perception of pain. These illnesses can be effectively treated and should be! Physicians of all specialties, including hematologists and oncologists commonly involved in the care of MDS patients are well aware of the impact of these psychiatric illnesses. At my institution, they often encourage their patients to seek treatment and will refer to our psychosocial oncology program. These support services are very common in cancer centers, and if you are having problems, talk with your physician.
Be Effective When Caregiving from a Distance

Being a caregiver is a difficult task, but being a caregiver and being hundreds or thousands of miles away can make this difficult task even more complicated. Remote caregiving for a loved one is possible, with the right planning and coordination.

Below are some tips to help make it work:

1. **Build a care team.**
   
   It’s difficult for one person to meet all the needs of a loved one who is ill. It’s important to form a caregiving team that includes family members, neighbors, friends, and, if needed, paid caregivers. Different people can be tasked to line up meals, provide rides to the doctor, and arrange grocery shopping. Be sure to tap into the expertise of each team member — one person may be good at handling bills, another might be an effective advocate for health care, and someone else may provide loving company.

2. **Plan your visits.**
   
   When preparing to visit, it is important to do some planning. Talk to the primary caregiver(s) and make sure they know you are coming, and ask them how you can help while you are there. Try to schedule your visit when the patient has an appointment with their doctor so you can get answers to questions you might have.

3. **Leverage technology.**
   
   When you are organizing care from a distance, using online resources and social networks can facilitate communication among team members and the patient. Consider setting up a page on Facebook (www.facebook.com), CaringBridge (www.caringbridge.org), or Lotsa Helping Hands (www.lotsahelpinghands.com) to encourage conversation among care team members and to serve as a virtual information exchange. Also, take advantage of mobile apps with medical records and electronic calendar reminders to help you track your tasks. You can also communicate through video calling with programs as Skype or Facetime.

4. **Care for yourself.**
   
   Being a caregiver can be demanding and time-consuming, especially when you are helping out from a distance. When you are far away, there can be added worry from the uncertainty of the situation. Stress can negatively affect your health, well-being and ability to help. Remember to schedule regular time for what’s important to you — exercising, socializing, or just relaxing. Find out your company’s policy regarding caregivers. There may be benefits that can help ease your situation, such as flextime policies or job sharing to free up your schedule. Check out caregiving support services and support groups. There’s comfort in knowing others are experiencing the same ups and downs as you.

And don’t forget, we are here for you!
Call (800) – 747-2820, option 1, and speak with our patient educator about connecting with other caregivers through our one-to-one Support Connection network, and view information for caregivers at www.AAMDS.org/Caregivers.
“I’ve found a family,” explains a Community of Hope member.

Communities of Hope are volunteer-led local groups, working together with AA&MDSIF staff, connecting patients and families with each other. AA&MDSIF continues to build 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

Each community will look slightly different from another – there’s no one size fits all approach. It’s up to the members to decide how they want their group to function. The different types of groups that can be established include:

- 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.
- Enhanced patient support groups: These groups 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 focused groups: These groups join together to organize and participate in a specific fundraising and awareness event in their community.
- Advocacy and action-oriented groups: These groups work together in advocacy efforts to their elected representatives to help carry out AA&MDSIF’s advocacy work.

Make your Community of Hope meet the needs of its members.

In building a Community of Hope, you can 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!

Join a Community of Hope – a current list can be found on our website. Or, if you would like to start a Community of Hope in your town, contact Community Development Officer Martha Crews at crews@aamds.org or (301) 279-7202 x103.

LET THE AA&MDSIF SUPPORT CONNECTION HELP YOU

Because bone marrow failure diseases are so rare, it is difficult to get connect with others who are dealing with issues similar to your own. We can help by putting you in touch with other patients and families who know what you are going through.

Our one-to-one Support Connection network consists of volunteers, patients, caregivers and family members, who are willing to listen and offer comfort and support. Our volunteers know the importance of networking with other patients, spouses, family members and loved ones. Speaking with a Support Connection volunteer is a great way to gather information and receive emotional support.

If you would like to talk with a Support Connection volunteer, please call (800) 747-2820, and select option 1 or email clark@aamds.org, to help you get connected.
Meet Stephanie Cadell

Stephanie Cadell volunteers for AA&MDSIF in many ways: she leads a Community of Hope support group, and she organized a Hope, Steps & A Cure Walk. As Stephanie says, “I lost my Dad to MDS in 2011. It has become my goal to raise awareness and funds for research so that in the future, people don’t have to suffer with any of these bone marrow failure diseases.”

Through her leadership, the Phoenix Community of Hope support group is going strong. The group meets monthly to support one another, share thoughts, and learn. These participants have grown close, and they consider themselves a family, even visiting one another in the hospital or giving each other rides to treatments. It was important to Stephanie to get involved and give back by stepping up to lead the group.

And when she felt that more was needed to be done in her Phoenix community, she decided to organize a Hope, Steps & A Cure Walk in memory of her dad, in memory of other patients, and in honor of those patients and families living with a bone marrow failure disease. Through her leadership and dedication, she secured a beautiful walk venue, sought out corporate sponsorships, and spread the word of the walk so that others in the community would learn about the event and bone marrow failure disease.

The first Phoenix Hope, Steps & A Cure Walk, held on February 2, was a huge success with 13 walk teams, over 150 registered walkers, and over $20,000 raised.

AA&MDSIF is greatly appreciative of Stephanie Cadell’s volunteer work of leading a successful Community of Hope and organizing a fantastic walk. Stephanie is looking forward to planning next year’s Phoenix walk!

Volunteer Vibe

Establish a named research fund or contribute to the Research is Hope Fund

OR

Support:

• Patient and Family Conferences, online learning and other resources

• Communities of Hope that offer strength in numbers

• Special events that increase awareness and make a difference

• Scholarships for students affected by bone marrow failure diseases

• Or, create a legacy for yourself or a loved one through a bequest in your will or estate.

You may make your gift online at www.aamds.org/DonateNow or by mail at:

AA&MDSIF
100 Park Avenue, Suite 108
Rockville, MD 20850
301-279-7202

Thanks you!
I realized something wasn’t right when I was out of breath from simply walking up a hill to get to class during my senior year in college. I had been an active fencer since high school, and it didn’t make sense that walking up a hill would give me so much trouble. I visited my doctor after graduation, and I was put in the hospital the next day. My nurse came in and told me, “We are paging the doctor who does bone marrow biopsies to come back. He left to go home for the weekend, but I don’t think you can wait until Monday.”

It was a scary thought that I may be seriously ill, but I had no doubt that I was going to get through it. I would like to say that I was so confident because I’ve always been a stubborn fighter. In reality, I was just in denial that anything could be seriously wrong with me when I was only 21!

One month later, I was finally diagnosed with PNH. The transition from college to working full time as an information systems security professional was especially difficult while dealing with anemia and fatigue. I started on prednisone and my blood counts went on roller coaster rides – a minor cold would make my counts plummet. I had my first hemolytic (abnormal breakdown of red blood cells) episode with dark urine while I was over 2,000 miles away from home on a trip for work. I frantically called my doctors who increased my prednisone dosage to get through the trip. It was becoming obvious that staying on prednisone would not be a long-term solution (plus I hated the chipmunk cheeks). I started eculizumab (Soliris®) soon after and while my counts have never gone up to normal (my hemoglobin hovers around the 9s and normal for females is 12-16), they have held stable, so I could relax and adjust to life with PNH.

I had stopped fencing after college because of the combination of PNH and a full time job, but my favorite sport was always in the back of my mind. Three years after I was diagnosed with PNH, I stepped into a fencing club, and I immediately felt at home. I listened to the familiar sounds of fencing blades clashing, and I couldn’t wait to jump in. I made up my mind to try to fence regularly again and make it back to competitions. Getting into the rhythm of going to practice regularly turned out harder than I expected. It was always tempting to skip a practice because I felt tired. I learned to force myself to just get out of the house. Staying hydrated and getting enough sleep were also instrumental in getting my body to adjust to the new routine. Eventually I found that I actually have more energy when I am staying active.

Six months later, I started going to competitions again. Every competition has brought joy and frustration. Joy, because I am able to compete in the sport I love despite having PNH. Frustration, because constantly being out of breath is a reminder that I have PNH and my stamina will never be the same. Initially, handling the frustration was a bit of a struggle, but I realized how lucky I am just to be able to fence. I came to accept the frustration and embraced the joy. It has been a wild journey with PNH, and it molded me into the person I am today.

In addition to fencing again, I also added new experiences in my life since I was diagnosed with PNH. I have been a committee member with the PNH Foundation since the end of 2011. I became involved because I loved their mission and wanted to help other patients get access to a specialist and receive all the information about PNH that they need.

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I also went fishing on a boat and tried out horseback riding, and I’m looking forward to traveling to new places. PNH hasn’t stopped me from enjoying life, and I plan to keep enjoying it for a long time to come.

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Fact Sheets
- 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
- Online Learning Center

To order a patient packet, call (301) 279-7202 x116, or order online at www.AAMDS.org/Info.

* Available in French, German, Italian, Portuguese, Spanish  ** Available in Spanish

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**
- Your Guide to Understanding MDS Drug Therapies*

The Online Learning Center has information for patients and families on treatment options and issues, and living well – topics including fatigue, nutrition, emotional coping, and caregiving. Learn at your own pace and in the style that suits you best!

EXPLORE
- Live and Archived Webinars
- Video Interviews with Experts
- Prerecorded Webcasts
- Interactive Learning Modules

IN PERSON

Phone Support for Personal Attention
Please contact our Patient Educator at (800) 747-2820, option 1, or by email at info@aamds.org, for answers on a wide range of questions, including information on treatment options, clinical trials, financial resources, and more.

Support Connection
The Support Connection 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.

Call now!
To connect with a Support Connection volunteer, call (800) 747-2820, option 1 and speak with our Patient Educator, who will match you with one of our volunteers. You can also email info@aamds.org.

Aplastic Anemia & MDS
INTERNATIONAL FOUNDATION

100 Park Avenue, Suite 108 • Rockville, MD 20850
Phone: (301) 279-7202 or (800) 747-2820
Fax: (301) 279-7205
help@aamds.org • www.AAMDS.org
No INterNet access at home?

Internet-connected computers are found in many locations, including:
- Retirement homes
- Public libraries
- Apartment community rooms
- Senior centers

And it’s almost certain you know someone (relatives, neighbors, friends) who is connected!

Connect with us at www.AAMDS.org!

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

Learn More Inside

- Read about our new global resources, [pg. 1]
- Find out why AA&MDSIF is the ‘resource of choice’ for patients and families, [pg. 6]
- Learn about how to be an effective caregiver from a distance, [pg.11]
- Meet one of our dedicated volunteers who is keeping busy managing more than one volunteer activity! [pg. 13]

Take Action

- Support AA&MDSIF today. Contact vehar@aamds.org, or call (301) 279-7202 x113 www.AAMDS.org/DonateNow
- Include AA&MDSIF in your will – join the Legacy Society: Guardians of Hope. Contact spears@aamds.org or call (301) 279-7202 x122.
- Establish a named research fund or contribute to the Research is Hope Fund. Contact witt@aamds.org or call (301) 279-7202 x104.
- Help create a Community of Hope in your area. Contact crews@aamds.org or call (301) 279-7202 X103

Connect with us at www.AAMDS.org!

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Return this page with your comments, email update@aamds.org or call (301) 279-7202 x105.