Insider
Aplastic Anemia & MDS International Foundation

New e-resources for patients!
AA&MDSIF presents two new ways to help patients learn about and manage their disease.

What is MDS? iPad app
Using this iPad app, patients and their families can learn about MDS in several ways:

• Watch the What is MDS? video that provides a basic animated description of the disease
• Read, listen to, or view the Frequently Asked Questions About MDS interview with an expert
• Read the extensive disease-related content within the app, including information on why MDS results in low blood counts and the associated symptoms

Learn more about this iPad app and watch the What is MDS? video at www.AAMDS.org/MDSapp. The app can be downloaded from www.apple.com/itunes.

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Patients and Families Learn from AA&MDSIF Coverage of Research

Through surveys, group and individual conversations, social media, and other methods, AA&MDSIF has learned that many of our constituents closely follow research – approximately twenty percent follow it weekly; thirty-six percent monthly; and sixteen percent quarterly.

With this in mind, we are focused on presenting this information in convenient formats to keep patients and families updated with the latest information in basic research and current clinical practice. In this edition of the Insider, we’ll show you some of the ways we are delivering on our commitment to keeping patients, families, and caregivers updated with current research.

What is this research information and where is it found?
Research in bone marrow failure diseases is occurring around the world primarily in academic, corporate, and government settings. It is then presented at professional society meetings and smaller, more specialized conferences, published in scientific journals, and shared online. Published research is indexed in the National Library of Medicine’s MEDLINE database, and accessible through its PubMed interface on the Web. (www.pubmed.gov)

How does AA&MDSIF present research to patients and families?
With advice and input from the AA&MDSIF Medical Advisory Board, and opinion leaders in bone marrow failure diseases, research topics are selected and presented to patients and families in print, online, and in person.

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Sometimes the best gifts we receive don’t come wrapped in pretty paper or tied with colorful ribbons. They come as a true gift, given without expectation of anything in return, given selflessly.

In my job, I am very fortunate to see - and receive - many of these types of gifts, so let me share some of MY favorite things. Maybe they are YOUR favorites too:

• Doctors who take a few extra minutes to answer another question or make sure you understand what was just discussed, even if they are already late to their next appointment;
• The initiative a young woman took to study the critical medical and social issues that adolescent patients face transitioning to young adulthood, and then sharing what she found;
• Hundreds of volunteers giving thousands of hours to start a Community of Hope in their area or organize fundraising events or call another patient who needs someone to talk to;
• Families who plan a legacy gift to support research to find better treatments or cures for the diseases that have touched them and their loved ones;
• The experts who give up their weekends or free time to speak at our conferences or lead a webinar or write and review our materials so that we and you have the very best information;
• Nurses and technicians who know you are hurting or frightened and take extra care with a needle and offer a comforting word;
• Conference participants who take packets of information to their doctors so that newly diagnosed patients will know how to find answers, support, and hope more easily than they did;
• The confidence you show in us and our organization to serve you and provide you with the information, materials, and services you need;
• Staff members who take the extra time to be sure you get what you need today and who always remember to “wear the patient’s sneakers”;
• The kind words of “Thank You”;
• And about a hundred more things.

These may not be “raindrops on roses and whiskers on kittens”, but they are a few of my favorite things, these gifts without ribbons. So, as we reflect on the gift giving seasons, take a minute or two to think about the gifts you receive and the gifts you give. Not only the ones in pretty paper, but the ones that come from the heart.

Thank you for the gifts you have given us and given me.

Stay well,

John M. Huber
Executive Director
Aplastic Anemia & MDS International Foundation Receives Celgene Innovation Impact Award

AA&MDSIF has been named a recipient of a new Innovation Impact Award from Celgene Corporation for its development of the MDS Clinical Research Consortium.

About the Award

Inaugurated this year, the Celgene Corporation Innovation Impact Awards program recognizes effective, innovative, and successful initiatives of U.S.-based not-for-profit organizations addressing the needs of patients, caregivers, and healthcare providers in today’s challenging healthcare environment in one of two therapeutic areas: hematology or oncology. Winners receive a $100,000 cash grant and are invited to participate in an Innovation Experience workshop.

“Even from the earliest stages, we believed that the MDS Clinical Research Consortium was an innovative, game-changing project that was perfectly aligned with our strategic plan. It’s exciting—and humbling—to see that others are seeing the project in that same way,” said AA&MDSIF Board Chairman, Kevin Lyons-Tarr.

Submissions for the award represented a wide range of approaches to support the hematology and oncology communities, and had to address a defined challenge faced by the target population. An independent judging panel that included innovation, healthcare, and advocacy experts identified the award recipients from a pool of applicants.

About the Clinical Research Consortium

Established in July 2012, the MDS Clinical Research Consortium is an unprecedented collaboration of six of the largest MDS centers in the U.S., designed to undertake unique studies and trials to advance treatments and improve outcomes for patients with myelodysplastic syndromes (MDS). It fills a major gap in MDS-related clinical research in the U.S. by providing a new “critical mass” of patients and patient data to support the evaluation of promising new compounds, epidemiological studies, and translational studies leading to new classifications, treatments, and procedures.

The five-year, $16 million initiative is sponsored by AA&MDSIF and supported by the Edward P. Evans Foundation. The programs are located at the Cleveland Clinic Taussig Cancer Institute, the Dana-Farber Cancer Institute, the MD Anderson Cancer Center, the H. Lee Moffitt Cancer Center & Research Institute, the Weill Medical College of Cornell University, and the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins.

“We are very pleased and honored to be selected for this new and prestigious award,” stated John Huber, AA&MDSIF Executive Director. “We deeply appreciate the support of the Evans Foundation and our six consortium partners who have made this innovative, exciting and high impact program possible.”

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Researchers and Physicians to Convene in March 2014!

With exciting new developments in the treatment of aplastic anemia, MDS, and PNH, and new insights into the molecular pathogenesis of these diseases, AA&MDSIF is producing the Fourth International Scientific Symposium as a forum for attendees to explore opportunities for collaborative research.

This meeting joins physicians with researchers studying the immunology and cell biology of bone marrow failure disease to share recent research results, discuss current areas of controversy, and propose specific priorities for basic and clinical research.

The symposium will address the most up-to-date issues in each topic and encourage participant interaction. Each session includes presentations by experts in the field followed by open discussion. For a complete agenda, poster session information, and registration for the symposium, visit www.AAMDS.org/Symposium2014.

AA&MDSIF Closes 2013 With Events Around the U.S. for Professional Education

AA&MDSIF packed the last half of 2013 with live events designed for researchers, physicians, nurses, and nurse practitioners.

- **Baltimore:** September’s Bone Marrow Failure Disease Symposium updated attendees on the most recent research related to the diagnosis and clinical management of bone marrow failure diseases, with a focus on translation of research findings into clinical practice and improved patient care.

- **Milwaukee and New York City:** Treating Low-Risk and High-Risk MDS. These two complimentary dinner and educational programs for nurses and nurse practitioners took place in October and November, spanning topics including pathophysiology, diagnostic criteria, classification and scoring system, and treatment strategies.

Patients and families attended a morning meeting the next day, where the same presenters delivered a program designed for the patient audience.

- **Chicago:** October’s third MDS/MPN Rounds at Rush University Medical Center was an intensive symposium of clinical challenges on the treatment of myeloproliferative neoplasms and myelodysplastic syndromes, featuring case presentations and interactive discussions.

- **New Orleans:** Co-sponsored with the Cleveland Clinic, the December American Society of Hematology (ASH) 2013 Satellite Symposium, “Turning the Tide Against MDS and Other Bone Marrow Failure Syndromes” focused on an in-depth and up-to-date review of research related to the biology, prognosis, and therapeutic management of MDS and other bone marrow failure syndromes.

Information for Patients from ASH 2013!

The 2013 American Society of Hematology Annual Meeting (ASH) in New Orleans December 6-10 featured numerous presentations on bone marrow failure diseases from leading researchers. AA&MDSIF has identified the reports on aplastic anemia, MDS and PNH that are most relevant to patients and their treatment team, and this information is available to patients in print and online in the following ways:

- Video interviews with AA&MDSIF Medical Advisory Board members and grantees
- Summaries of key research studies written in lay language
- Webinars in early 2014 with experts discussing the most important research news for patients

Visit www.AAMDS.org/ASH2013 to view the video interviews and read or download the research summaries for patients.
For over 20 years, AA&MDSIF has provided research grants totaling over $3 million to an international group of more than 50 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.

Andrew Dancis, MD
Associate Professor, Medicine
Hematology/Oncology
University of Pennsylvania
Funded By: Research is Hope Fund

SF3B1 mitochondrial phenotype in myelodysplasia as a therapeutic target

A characteristic finding in blood cell precursors of some individuals with myelodysplasia (MDS) is the ringed sideroblast, a cell that accumulates large amounts of toxic iron in mitochondria. Recently, the presence of these abnormal mitochondria has been correlated with mutations of the splice factor SF3B1. We plan to investigate the mitochondria of these cells with perturbed SF3B1, aiming to gain insight into mitochondrial causes of MDS. This may point to new therapies.

Hideki Makishima, MD, PhD
Project Staff, Department of Translational Hematology and Oncology Research
Cleveland Clinic Taussig Cancer Institute
Funded By: The PNH Research and Support Foundation

Clonal Architecture in PNH: Somatic genetic defects facilitating clonal expansion

PNH is a disease in which a mutation in the gene called PIG-A is acquired in the stem cells (mother cells of all blood cells) in the bone marrow of patients. As a result, the blood cells produced by this stem cell are defective. While previous discoveries of the PIG-A gene mutation has helped to explain the symptoms in the disease, it remains unclear how PIG-A mutation makes the PNH stem cells outcompete healthy stem cells. In this project, we propose to apply a very efficient sequencing technology to examine all genes in PNH stem cells to see whether additional mutations will explain how PNH develops. In the initial experiments, we have identified such additional mutations. They may help to devise treatments to eradicate PNH stem cells from the patient’s bone marrow.

Rosario Notaro, MD
Deputy Director
Core Research Laboratory
Istituto Toscano Tumori, Florence, Italy
Funded By: Amber Lynn Wakefield Research Fund

The role of GPI-reactive T cells in the pathogenesis of acquired aplastic anemia

Aplastic anemia and paroxysmal nocturnal hemoglobinuria (PNH) are two serious blood disorders that share one important feature: the bone marrow cannot always keep up with the body's needs for blood cells. This means that there may be anemia, low white cells (particularly neutropenia, entailing the risk of infection), or low platelets (with risk of bleeding). Recently, we have analyzed in depth a type of lymphocyte cells called T cells in patients with PNH. We have found that they have an excess of a very rare subset of T cells that are able to recognize a specific glycolipid molecule (a molecule that contains both a sugar moiety and a fat moiety) – we have called them GPI-reactive T cells. We now plan to investigate whether GPI-reactive T cells are also increased in aplastic anemia.

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Research

2013 Research Grant Recipients (continued from pg. 5)

Our findings would corroborate the notion that these cells are prime movers of the disease and therefore may make it possible to develop new forms of treatment.

Eirini Papapetrou, MD, PhD
Assistant Professor of Medicine
Department of Hematology
University of Washington

Funded By: Research is Hope Fund

Modeling 7q-MDS with human induced pluripotent stem cells

Progress in understanding the etiology and effective treatment of MDS is currently hampered by the scarcity of tools to study this disease. Our goal is to harness cutting-edge human pluripotent stem cell and genetic engineering technologies. We plan to establish new models of an MDS subset characterized by loss of chromosome 7 material and use them as a novel platform to identify genes on chromosome 7 that are critical for this disease. These models should provide a powerful resource to the MDS community to investigate the cell biology, molecular pathogenesis, and genetic basis of MDS, and identify new therapeutic targets and perform drug screens.

Akiko Shimamura, MD, PhD
Associate Professor of Pediatrics and Director, Marrow Failure/MDS Clinic
University of Washington and Fred Hutchinson Cancer Research Center

Funded By: Torry Yahn Research Fund

Genetic predictors of response to immunosuppressive therapy for aplastic anemia

Currently, aplastic anemia is treated with immunosuppressive therapy (IST) or a bone marrow transplant. Approximately 30% of aplastic anemia patients treated with IST are refractory to treatment or develop myelodysplastic syndromes (MDS) and leukemia.

Tests to predict which patients will fare poorly to IST would inform upfront treatment decisions. We will utilize cutting edge genomic technologies to screen for genetic markers predictive of poor outcomes with IST. This study will develop a novel diagnostic tool for aplastic anemia to guide treatment decisions with the goal of improving patient survival.
Patient Education

New e-resources (continued from cover)

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!

Research Information from AA&MDSIF (continued from cover)

IN PRINT

• Research Summaries for Patients: Important research in bone marrow failure is first announced and presented at major medical and scientific society meetings. Our Medical Advisory Board selects the presentations that will be of most interest to patients. They are next summarized in lay language, and some feature an additional commentary on specifically what this research means for patients. The contents of these summaries are also addressed in a later webinar.

• Our Researchers Report: AA&MDSIF funds research on approved projects every year. Research grant recipients are required to write a progress report after their first year has been completed and a summary report after the second (and final) year.

ONLINE

• The Latest Research section on our Web site, seen at www.AAMDS.org is a searchable group of research abstracts selected from PubMed.gov. New articles are added each week.

• Research Reviews, also accessed at www.AAMDS.org/ResearchReview, focus on recent, important medical and scientific journal articles. These lay language reviews are written by hematology/oncology professionals who interpret and explain the often complex information in an original article for the benefit of patients and families.

• Interviews With the Experts is part of the AA&MDSIF Online Learning Center, (www.AAMDS.org/Learn), and some interviews are summaries of the state of research in a selected area.

• Webinars and Webcasts are also part of the Online Learning Center. Some webinars (live online events) and webcasts (prerecorded events) review and summarize recent research.

IN PERSON

• AA&MDSIF Patient & Family Conferences and other live events often feature research experts speaking directly to patients about the current state of research or a particular topic of interest, and their presentations are archived on the Online Learning Center.

In 2014, be sure to look to AA&MDSIF for more of the latest, relevant research in the formats that work best for you.
Can you briefly explain the four phases of a clinical trial and why these separate stages are needed?

Clinical trials in medicine are divided into four phases. Phase 1 trials are designed to test new medications, or combinations of existing medications, for the first time in human subjects. The goal of phase 1 trials is not to determine if a drug works against a particular disease, but to determine what doses are safe and tolerable to patients so that the best dose can be used in larger, more advanced trials.

Phase 2 trials involve more patients than phase 1 and can be more complicated. The goal in phase 2 trials is to determine whether a drug has efficacy. If it doesn’t seem to work, or if unexpected toxicity crops up, it is unlikely to be developed further.

Phase 3 trials are typically very large, multi-institutional trials designed to better understand how well a drug works compared to existing treatments. Phase 3 trials are often randomized where patients are given either the experimental drug or an alternative such as an already approved therapy or placebo in its place. A placebo is a treatment that looks just like the experimental treatment, but lacks the active ingredient. In double blind clinical trials, neither the patient nor the doctor knows whether the patient is getting the experimental therapy or the placebo. Phase 3 trials are designed to determine efficacy and to look for signs of toxicity that might not have been evident in the smaller, earlier phase trials.

Medications are often approved based on the results of phase 3 trials, although occasionally based on results of phase 2 trials if they show a dramatic degree of efficacy or treat a life-threatening condition with few alternatives.

After a drug has been approved by the FDA, they sometimes ask for what is unofficially called a “phase 4” trial. These are post-marketing studies that monitor a great number of patients receiving a treatment. The objective of these trials is to validate the results of earlier trials -- in particular, to confirm their results in a more real-world setting and looking for unanticipated toxicity. It’s rarely required, but in some cases, the FDA will approve a medication contingent on post-approval trials being done.

One person does not go through all phases of a trial. Each phase is a distinct study separated in time by quite a bit from earlier phases. In fact, if a later phase trial requires that a patient has never received this drug, or one like it, participants in the earlier trial would not even be eligible. There are some combined phase 1/2 trials where the first few participants help identify the ideal dose in the phase I portion and a larger group of participants receive that dose in the phase 2 portion.

Some clinical trials focus on a new drug, sometimes a combination of drugs, and sometimes a new use for an existing drug. How are these different approaches utilized?

Some trials focus on drugs never before tried in humans, some test combinations of approved drugs, while others are for an already approved drug used in a different setting. Here, the manufacturer wants proof that it works for another disease condition so that they can promote the product for this new indication. Keep in mind that physicians can prescribe an already approved medication for non-approved indications, which is referred to as “off-label prescribing,” but the FDA prohibits the manufacturer from advertising for the off-label use. Manufacturers may want to prove the drug works for a different purpose so they can actively promote it.

How do you advise patients who feel that clinical trials are just a last resort to be avoided if possible, or others who feel that unless they are in the group getting the drug(s) being tested that participation isn’t worthwhile?

This is a very important point. There has been some resistance to, and misunderstanding, of clinical trials. This results in fewer patients willing to consider participating in a clinical trial. Part of the misunderstanding is about the role of clinical trials. It’s true that there some are designed to enroll patients who have tried all other approved therapies and are left with few options. This is common in metastatic cancers, for example.
There are clinical trials available for almost every stage for every disease. There are ones for patients who have never been treated before in order to come up with a better first line treatment for them. Other trials are designed to treat patients who have tried just one or two prior therapies, but still might have other options available. The hope is to always offer something that might be better than the current standard of care.

An institutional review board comprised of doctors, nurses, clergy, and laypeople will scrutinize all clinical trials in their approval process to protect the rights and welfare of potential participants. This is not only for patient safety, but to ensure that the question being asked by the trial is a fair one—new for example, that patients who are randomized to the experimental drug are as likely to benefit as patients who are randomized to not get this treatment.

These are drugs that we may not have much experience with, and it is possible that an experimental drug does not work or has more toxicity than expected, and thus, could be worse than getting the standard of care or a placebo. The reason many people sign up for a clinical trial is for the possibility of getting the new drug being tested. But even if someone is randomized to the arm of the study that doesn’t get the novel agent, they will still receive more scrutiny and interaction with physicians. In general, patients in clinical trials do better than similar patients who are not. One reason for this may be the added attention they receive—namely more frequent evaluation and follow-up.

Can related diseases be studied in the same clinical trial?

This often happens in phase 1, where trials may not be limited to one disease type. The goal is to find the safe dose. So a cancer drug might be tried in patients with different types of advanced cancers, for example. In subsequent phase 2 or 3 trials, the drugs will be used in a more limited way, for example, just lung cancer patients, whereas in phase 1, it might have been tried in colon cancer and breast cancer patients as well.

What recently concluded trials for MDS have been interesting to you?

I have been interested in trials that combine already approved MDS drugs to see if they might work better than if used alone. For example, in a recently published Phase 2 trial of azacitidine (Vidaza®) and lenalidomide (Revlimid®) in combination, the toxicity was comparable to that of either drug used alone, but the number of patients who responded was greater than with either drug alone.

One of the problems with azacitidine is that fewer than half of patients get a positive response from the drug. With lenalidomide used in patients without the deletion 5q genetic abnormality, response rates also tend to be low. In combination, the response rates to these two agents were much better than predicted. The advantage here is that we do not have to wait for FDA approval to try this therapy in our patients when we have evidence that using this combination off-label may be safe and efficacious. This ability to quickly apply trial results in practice is one advantage of doing clinical trials with currently approved drugs.

What current MDS clinical trials are you watching or directly involved in that hold promise?

There is a follow up trial recruiting patients now that is based on the one I just described. This is a large phase 3 trial with three arms that compare azacitidine alone, the azacitidine and lenalidomide combination tested above, and azacitidine and vorinostat in combination.

This trial is designed to discover which is the best initial treatment for higher-risk MDS patients. If one treatment arm is shown to be superior to the others, this could be a major change in the standard treatment of higher-risk MDS. Some trials come into being based on the results of already completed trials. We learn a lot from small trials—these generate hypotheses that we can then test in larger trials. If we get to Phase 3, we really think we are onto something based on what we learned from earlier studies.

Have there been any unexpected failures or dead-ends that surprised you?

In general, the reality is that many new experimental drugs that look good in preclinical studies do not make it all the way to becoming an approved drug. They may seem promising in the beginning, but do either fail to work as well as we hoped or are found to have unexpected side effects. For example, HDAC inhibitors as single agents have not had the effect in MDS that we had hoped for. So now, some HDAC inhibitors, like vorinostat, are being tried in combination trials like the one I mentioned earlier.

What should MDS patients remember most about clinical trials?

If we look to the past at what clinical trials have done for medicine, in retrospect, the standard of care has always been replaced by something that was proven to be better in clinical trials. Patients who have been on clinical trials have gotten these therapies sooner and have benefitted from them before the general population does. Since we don’t have a cure for most MDS patients now, and since we need better therapies, then clinical trials are a very good way for getting access to newer, and potentially better therapies before they become available to the general public.
Patient Education

Social Media – Transforming the Way We Communicate

AA&MDSIF has a Facebook page (www.facebook.com/aamds), a Twitter account (www.twitter.com/aamdsif), and a YouTube channel (youtube.com/aamdsif). These social media tools are helping us connect with patients, families, and healthcare providers in new and innovative ways.

Patients connect with AA&MDSIF

Do you have a question about bone marrow failure disease? One way you can get answers is to call us and speak to our patient educator. Or you can email us or visit our website. Others will go directly to our Online Learning Center to watch a webcast or to use an interactive learning module. With social media tools, finding answers is as simple as a 140-character tweet or leaving a Facebook comment on our page. We’ll be there to answer your questions. These tools are helping us reach patients who might not otherwise have known about AA&MDSIF and its programs and services. We have recently connected with aplastic anemia patients who were using Twitter as an Internet search engine and found us by regularly searching for the keyword “aplastic” on Twitter.

Families support patients

Perhaps you want to support a family member with MDS by setting up a FriendRaising page through our website. You can then post a link to that page in our Facebook group. Also, for special occasions like #GivingTuesday, we highlighted the Debono family. They are raising money for the Matthew Debono Memorial Scholarship Fund in honor of their son, Matthew. It’s widely viewed on our Facebook page and has been a very successful fundraising and awareness project.

Connections with the research and healthcare communities

Well known medical institutions and physicians interact with us and follow us on Twitter. This includes Cleveland Clinic, Mount Sinai Hospital, and Boston Children’s Hospital. They are sharing our resources with their followers and we are sharing their research with our followers. We’re also reaching international healthcare providers! For example, we’re followed by the Saudi Society of Blood and Marrow Transplantation.

AA&MDSIF’s social media presence can help you connect with new resources and build a community. Find us on Twitter by searching for @aamdsif and on Facebook by searching for “Aplastic Anemia and MDS International Foundation”. See you online!

HOW CAN YOU BE INVOLVED IN CLINICAL TRIALS?

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

Clinical trials, also called research studies, are often conducted at university and government medical centers around the world.

If standard therapies have not helped you, or even if you are not satisfied with how well standard therapies work, you may want to explore whether you may be eligible for a clinical trial.

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. It is completely confidential to search, and registration or personal identification is not required.

ClinicalTrials.gov is a registry and results database of publicly and privately supported clinical studies of human participants conducted around the world. Learn more about clinical trials and about this site, including release history, policies, and links.

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Well known medical institutions and physicians interact with us and follow us on Twitter. This includes Cleveland Clinic, Mount Sinai Hospital, and Boston Children’s Hospital. They are sharing our resources with their followers and we are sharing their research with our followers. We’re also reaching international healthcare providers! For example, we’re followed by the Saudi Society of Blood and Marrow Transplantation.

AA&MDSIF’s social media presence can help you connect with new resources and build a community. Find us on Twitter by searching for @aamdsif and on Facebook by searching for “Aplastic Anemia and MDS International Foundation”. See you online!
Awareness

2013 saw AA&MDSIF increase its activities across all patient program areas. As the year comes to a close, we wanted to take a moment and share a look at our activities in the past year.

**Patient Support**
- Our Patient Educator received over 40 calls per week from patients and caregivers, and each week answered over 50 emails and responded to over 20 queries on Facebook.
- More than 850 information packets were sent to patients and families who contacted AA&MDSIF for the first time.
- Support Connection volunteers reached out to listen, provide encouragement, and share personal experiences with an average of 20 patients or family members each month.
- Our Communities of Hope support groups now meet regularly in 16 cities around the nation. 2 new Communities of Hope locations were added in 2013: Portland (Oregon) and Milwaukee, Wisconsin with 8 more in the process of being established.

**Patient Education**
- Over 20,000 users each month visited our website to learn more about bone marrow failure.
- Almost 1,800 registered for live webinars.
- The Online Learning Center had over 17,000 visitors.
- Nearly 800 patients and their family members attended our six patient and family conferences, with many more viewing webcasts and interviews produced at the conferences.
- Almost 130 links to relevant research articles were posted on the website.
- Scholarships were awarded to 15 students from 13 states through the Matthew Debono Memorial Scholarship program.

**Awareness and Fundraising**
- More than 300 volunteers helped to produce over 55 awareness/fundraising events (32 of which were new) raising over $205,000.
- The number of Facebook ‘likes’ increased by over 1,000, from approximately 3,100 at the start of 2013 to over 4,400.

In 2014, we plan to do even more! Whether you are a patient, spouse, caregiver, family member, or friend, newly diagnosed or have lived with a bone marrow failure disease for years, we have something for you. It's three things actually—**Answers, Support, and Hope.**

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**Aplastic Anemia, MDS, and PNH 2014 Regional Conferences**

**DIAGNOSIS, TREATMENT, AND MANAGING THE NEW NORMAL**

**Meeting You Where You Are**
- LOS ANGELES, CA | APRIL 5
- PHILADELPHIA, PA | MAY 17
- LOUISVILLE, KY | JULY 26
- DETROIT, MI | SEPTEMBER 20
- NEW ORLEANS, LA | OCTOBER 11
- MIAMI, FL | NOVEMBER 8

**Agenda**
(See Website for More Detailed Agenda)

- **7:30 - 8:30 AM** Check-in and Breakfast
- **8:30 - 8:50 AM** Conference Welcome
- **9:00 - 10:30 AM** Session A: Your Life Changing Phase of Diagnosis
- **10:30 - 10:45 AM** Break
- **10:45 - 12:00 PM** Session B: Your Life Changing Phase of Treatment
- **12:00 - 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

**Follow Your Disease Track**

Learn More! Visit
www.AAMDS.org/Conferences
Volunteer Vibe

Food and Fundraising – a Natural Combination for Kim Fernandes-Huff

Kim (left) and her friends at the 2013 sMAC Down, which raised almost $4,000!

AA&MDSIF volunteer Kim Fernandes-Huff is a very creative fundraiser! Each year, she coordinates two separate awareness and fundraising events – the sMAC Down and the Wing Fling. The sMAC Down is held in November, and it is a macaroni and cheese bake off with amateur and professional contestants vying for “The Big Cheese.” The Wing Fling, held in April, is a chicken wing cook-off, again with both amateur and professional participants. Both of these events are held at a local restaurant, The Beach House in Falmouth, Massachusetts. For an admission fee, participants can taste and vote – and enjoy entertainment and raffles.

Kim organizes these two events, along with great help from family and friends, in support of the Michael Fernandes Research Fund. Her brother Michael passed away from MDS in 2003, and the family established a research fund in his name. Kim says, “He had the most infectious laugh and silly sense of humor and made everyone smile. This fund is to honor him and to provide the hope and comfort he would want others to have.”

Kim and her family and friends have done an incredible job of raising awareness about MDS and bone marrow failure disease, raising over $28,000 for the Michael Fernandes Research Fund.

Organize a Fundraising Walk!

The Hope, Steps & A Cure Walk is a fun and festive walk that you can organize in your own community to raise awareness about bone marrow failure diseases while raising much needed funds for AA&MDSIF programs and services to help patients and families.

There is no “one size fits all”. You can design a walk that fits your interests and your timeline!

Learn more at www.AAMDS.org/Walk

Facts for life

National Donor Day – Donate Marrow, Blood, or Organize a Drive!

Have you or someone you love received a blood transfusion? Then you know how important those donations were to you and your loved one. January is National Blood Donor Month, and you can honor your loved one by donating blood or organizing a drive. It’s easy, painless, and a great way to support the cause and help save someone’s life!

The blood donation process is very simple and will only take about an hour. First, you will need to schedule an appointment with a local donation facility. The American Red Cross can help you find a donation facility near your home. On the day of your donation, be sure to bring a photo ID. The staff who register you will go over the donation process and answer any of your questions.

Staff will ask you questions about your past medical history, and they will also check your blood pressure, pulse, and temperature, and take a sample of your blood to check your hemoglobin level. Next, staff will clean the donation area on your arm and insert a brand new sterile needle. The actual donation only takes about eight to ten minutes. If you decide to donate platelets, plasma, or red cells, the donation process is longer and can take up to two hours.

Once the donation process is complete, staff will remove the needle and place a small adhesive bandage on your arm. After donating, it’s a good idea to eat a snack and have something to drink. Refreshments are provided by the donation facility. You will be able to leave the donation facility 10-15 minutes later and return to your normal daily activities. One important thing to keep in mind is that if your family member is considering a transplant and you have not been tested to see if you are a match – do not donate blood until after you have been tested and ruled out as a match.

For more information about donating blood or organizing a blood drive, contact the American Red Cross.
One day, our lives changed. Not totally, but enough to cause an adjustment and to open a new chapter. That’s the day I was diagnosed with MDS.

At the Start
My primary care physician was preparing for my annual physical in early January of 2010 and spent time the night before going over years of my blood records. I’d always been in the low range for platelet count. That extra investigative step, for which I was truly grateful, came on the recommendation of a hematologist/oncologist at Cape Cod Hospital. “Just a precaution,” he said. I was 65 at the time.

The hematologist and his nurse performed a bone marrow exam on the spot. And then came the diagnosis of MDS with isolated deletion 5Q—and an explanation of what MDS was and wasn’t.

Among my first questions was: Is MDS hereditary? With the answer of ‘no’ and the news that it was ‘acquired’, I breathed a sigh of relief for my son, Tim. Another of my questions was about treatment. I was told we’d “watch and wait” and get quarterly blood draws. Upon being told that there was no cure at present, I queried him on my life expectancy. He delicately surrounded his answer with ample wiggle room, but at least I knew it wasn’t imminent.

My hematologist offered referrals to either Sloan Kettering in New York City or Dana-Farber Cancer Institute in Boston for a second opinion. Location was important, and I went to Dana-Farber since I live in Sandwich, Massachusetts. I had another bone marrow procedure and a confirmation, but with a reference that I was categorized as “low-risk” and a promise that my stats would be entered into a database for research.

So our lives changed, but the externals didn’t. I told my wife, our son, and my siblings, but went to work the next day. In those early days, I was mostly numb -- trying to absorb the news and the ramifications.

The “watch and wait” and blood draws moved from quarterly to monthly to bi-weekly lasting until October of 2011, at which point my doctors agreed that it was time to begin a course of lenalidomide (Revlimid®).

It was then that I resigned my post running a local NPR radio station. My wife and I didn’t think it wise to continue a 50-60 hour work week with high stress and simultaneously try to adjust myself to medications and my increasing fatigue. That proved difficult as I had always worked, and in many ways had defined myself by my work.

Several months later, I was also diagnosed with idiopathic thrombocytopenic purpura, (ITP) a low platelet disorder. So I was then faced, and continue now, with weekly CBC draws and with an injection of romiplostim (Nplate®) the next day.

I consider myself, now at age 69, very lucky as those medications were not even on the market a few years ago. I’m very lucky to have really good doctors. My doctor at Dana-Farber and his crew are considered among the best in the world, and my hematologist has no hesitation in conferring with him. And I’m also very lucky to have a supportive wife who has her own health concerns.

Yet, we are all inevitably and ultimately in charge of our own health care decisions. I continue to stay abreast of developments through Web sites and emails. I always have a typed list of questions for my appointments. I eat properly, exercise regularly, and try to stay optimistic.

Looking Forward With Courage
While there’s much we cannot control with MDS, we can choose to act and we can choose to cultivate our spirit and positive energy. I have the stamina and the desire for volunteer board work with two organizations in the community. I’ve found that putting the focus on someone else and something else helps my own attitude about my illness. And if I’m upbeat, so are others.

On a daily basis, I try to organize, to prioritize, and to know my limits. A nap isn’t a bad thing. Who knows about the future? But for now, I’m living as fully, as light-heartedly, and as purposefully as I can.
Patricia and Vincent Geczik Legacy Gift to Fund PNH Research

“Mom always joked about ‘loaves and fishes,’” said Dana Consoli. “She never turned anyone away. Everyone was welcome at our table.”

It was that essential commitment to helping others that created a legacy for patients and families with bone marrow failure diseases.

Dana Consoli is the daughter of Patricia and Vincent Geczik, who remembered the Aplastic Anemia & MDS International Foundation with a special gift in their estate. Their gift will support AA&MDSIF’s efforts to find better treatments and perhaps a cure for paroxysmal nocturnal hemoglobinuria (PNH), Dana’s disease.

“We were a normal, everyday, middle-class family. My parents worked very hard, and they saved and saved and saved. My siblings and I never wanted for anything, and they instilled in us a great work ethic and great values,” said Dana. The family first lived in New York and then New Jersey. Vincent, who died in 2011, worked for UPS. Patricia, who passed away in 2013, used her creativity as an artist and also in a flower shop.

Working with an estate planning advisor, the Gecziks sought to use the proceeds of their life’s work to help “eradicate PNH.” That’s when they found AA&MDSIF. “They were tortured by the fact that I had PNH and had to edit my life,” explained Dana. “They knew my struggle, but they also knew that I was not going to let PNH define me or my life,” she added.

Today at 48, Dana has three “miracle” children, including a set of twins, and she enjoys a very active lifestyle. She’s recently begun off-road bicycling. And she is equally committed to her parents’ goal. “Ten years ago, I was very ill and could never have predicted that I’d be in such a good place. But it’s not enough. We need to do more for the patient, beyond immediate care and treatment. We need to continue this [work]. It’s a lot of responsibility making sure it is not squandered and lasts for generations. We want the legacy that my parents created to have a positive and lasting impact. We don’t know what that looks like yet, but that is the goal.”

“The gift that Dana’s parents have made is truly remarkable,” said AA&MDSIF Executive Director, John Huber. “The new research it will support, the discoveries it may launch, and the hope it engenders – that is their legacy.”

You can help provide answers, support, and hope for thousands of patients and families living with aplastic anemia, myelodysplastic syndromes (MDS), paroxysmal nocturnal hemoglobinuria (PNH), and related bone marrow failure diseases all year long!

Join us as a monthly giving partner in 2014.

To get started today:
- You determine the amount of your monthly donation
- We charge your credit card each month
- At year end, we send an annual giving statement for your tax purposes

Thank you!

AA&MDSIF is a 501 (c) (3) organization, Federal Tax ID #52-1336903. Gifts to AA&MDSIF are tax-deductible to the fullest extent of the law.
**IN PRINT**

**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 Spanish  ** Available in Spanish and French

**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)

**ONLINE**

Aplastic Anemia & MDS International Foundation
Online Learning Center
www.AAMDS.org/Learn

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
- Prerecorded Webcasts
- Video Interviews with Experts
- Interactive Learning Modules

**AA&MDSIF ON SOCIAL MEDIA**

- Join the AA&MDSIF Facebook Community
  www.facebook.com/aamds
- Follow Us on Twitter
  www.twitter.com/aamdsif
- Learn From Our YouTube Channel
  www.youtube.com/aamdsif
- Meet Up On
  Marrowforums

Marrowforums hosts free online discussions

**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.

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help@aamds.org • www.AAMDS.org
Address or email change? Prefer to receive only email? Want to be taken off our mailing list? Return this page with your comments, email update@aamds.org or call (301) 279-7202 x105.

Checklist for Winter 2014

Learn More Inside

- Read about our new e-resources for patients and the various ways AA&MDSIF brings relevant research directly to patients and families. [pg.1]
- Meet the 2013 AA&MDSIF Research Grant recipients. [pg.5]
- Read about the basics of clinical trials. [pg. 8]
- See AA&MDSIF in 2013, “by the numbers.” [pg. 11]
- See how social media is changing the way AA&MDSIF communicates with you. [pg. 10]

Take Action

- Learn how the generosity of one family will help PNH patients for years to come. [pg. 14]
- Dedicate a day on our 2014 Calendar of Hope. www.AAMDS.org/Dedication
- Include AA&MDSIF in your will and become a Guardian of Hope. Contact spears@aamds.org or call (301) 279-7202 x122.
- Help create a Community of Hope in your area. Contact crews@aamds.org or call (301) 279-7202 x103.

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

Connect us at 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!

Contact Us

help@aamds.org  (301) 279-7202 or (800) 747-2820