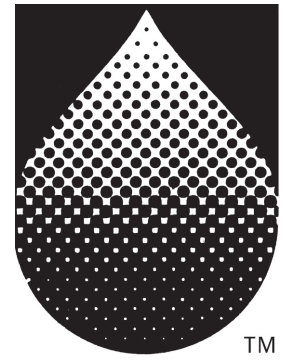


Aplastic Anemia & MDS International Foundation, Inc.

Fighting Bone Marrow Diseases through Patient Support & Research Since 1983



Patient Information

Our expert Medical Board will answer your questions and provide information on any issue. Call us - we are standing by ready to help you!



Ask the Experts

Go to www.aamds.org to review the archive of patients' questions which have been answered by our distinguished panel of medical experts. You can also personally ask your own question of these experts at this site.



Patient Clinical Trials

We give you an updated list featuring doctor's name, phone number and explanation of treatment protocols.



Patient Travel Fund

Families traveling to clinical trials can receive up to \$500 for travel expenses. Contact our office to receive an application form.



Global Network of Volunteers

You are not alone - patients and families will share their treatment experiences with you. Call us to make a friend!



Patient Registry

Statistics on these diseases seem to be as rare as the diseases themselves. Please fill out our Patient Registry Form for surviving or deceased patients. The more data we collect, the more data we can use for research.



You Can Help

Please donate your blood & platelets, have your bone marrow tested, and financially donate to the AA&MDSIF's research & support efforts.

New Medicine Approved for All Subtypes of Myelodysplastic Syndromes

Dacogen® becomes third treatment approved in the United States for MDS

MGI-Pharma/SuperGen announced the U.S. Food and Drug Administration's (FDA) approval of Dacogen® (decitabine) for treating myelodysplastic syndromes (MDS). Dacogen® is a member of a new class of drugs called hypomethylating agents. It is thought to work by restoring normal growth and differentiation of bone marrow cells.

"Myelodysplastic syndromes are a group of rare diseases where the body does not produce enough healthy, properly functioning blood cells; patients often become transfusion dependent with MDS. The FDA's approval of Dacogen® offers patients with all subtypes of MDS hope for an improved quality of life and better health," stated Marilyn Baker, AA&MDSIF President.

Only a few years ago, no drug therapies were approved for treating MDS. Until recently, patients were dependent solely on supportive care with blood transfusions and the use of growth factors such as Procrit® and Epogen® (epoietin alfa), or Aranesp® (darbepoetin alfa) to stimulate the production of red blood cells. Neupogen® (filgrastim, G-CSF), a medicine to stimulate white blood cell production, may be prescribed also. The use of growth factors is still considered a standard approach for treating MDS, especially in its milder forms, but the approval these new medicines offers patients' options.

Dacogen® is the third medication approved by the FDA for treatment of MDS. In 2004, Vidaza® (azacitidine), a hypomethylating agent very similar to Dacogen®, became the first FDA-approved treatment of all subtypes of MDS. In December 2005, Revlimid® (lenalidomide) was approved for low-to-intermediate MDS with the specific chromosomal deletion of 5q. Revlimid® is an immunomodulatory drug (IMiD), a new class of drugs that can modify or regulate the functioning of the immune system. These three treatments offer promise and options for patients with myelodysplastic syndromes.

Dacogen® is an injectable medication for treating patients with MDS, including previously treated, untreated, de novo (of unknown cause), and secondary MDS of all French-American-British (FAB) subtypes (refractory anemia, refractory anemia with ringed sideroblasts, refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, and chronic myelomonocytic leukemia) and Intermediate-1, Intermediate-2, and High-Risk International Prognostic Scoring System groups.

In clinical trials, the safety and effectiveness of Dacogen® was demonstrated in a randomized, controlled trial in which patients received either Dacogen® or the best standard therapy and in two nonrandomized studies in which all of the patients received Dacogen®. The new drug was evaluated in 268 patients.

Approximately 22% of patients in the three trials had complete or partial responses to Dacogen®. Responses consisted of complete or partial normalization of blood counts and of fewer immature cells in the bone marrow. In responders, the need for transfusions was eliminated during the period of response.

The most common side effects of Dacogen® reported in clinical trials included neutropenia (low white blood cell count), thrombocytopenia (low platelets in blood), anemia, fatigue, fever, nausea, cough, bleeding in the skin, constipation, diarrhea, and hyperglycemia (high blood sugar). Dacogen® is expected to be available for distribution during the second quarter of 2006. ♦

Legacy of Hope



Robert F. Carroll, Ph.D.

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

In 1991, at the age of 57, Bob was diagnosed with Myelodysplastic Syndromes and given only about two years to live. He often said that moment changed his life because it was in that minute that he committed himself wholeheartedly to make life better for other people. *"I consider MDS to truly be a gift. It is my gift because it has taught me that relationships with others are what are important in my life – nothing else."* It is this beautiful spirit and dedication to helping people that enabled Bob to beat his disease for so many years.

While the details of his tireless acts of generosity and inspiration were unknown to many of us, the spirit that animated them is one we witnessed – and were moved by – every day.

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

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

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

- ◆ The *Robert Carroll Legacy Fund* is dedicated to Bob's passion for helping patients and his devotion to our mission to our mission of leading the fight against bone marrow disease.
- ◆ The *Robert Carroll Legacy Fund* will raise money to increase patient support, advocacy, and research.
- ◆ The *Robert Carroll Legacy Fund* will help save lives, help families, and give hope to thousands around the world.

*Bob's spirit will live on in the AA&MDSIF
as well as in each of us that carry his love and hope forward.*

Recently Published Articles

Listed below are a few recently published journal articles indexed in the National Library of Medicine database MEDLINE: www.pubmed.gov. There is no fee associated with searching this database and no limit to the amount of searches that you may make. To obtain complete articles, contact your public or hospital library. These articles are listed for general information purposes only.

Aplastic Anemia

Sepulveda E, Brethauer U, Rojas J, Le Fort P. Oral manifestations of aplastic anemia in children. *J Am Dent Assoc*. 2006 Apr;137(4):474-8.

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Solomou, Elena E, Keyvan Keyvanfar, and Neal S. Young. T-bet, a Th 1 transcription factor, is up-regulated in T cells from patients with aplastic anemia. *Blood*. 2006 May 107(10):3983-91.

MDS

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Vey N, Bosly A, Guerci A., et al. Arsenic trioxide in patients with myelodysplastic syndromes: a phase II multicenter study. *J Clin Oncol*. 2006 Jun 1;24(16):2465-71. and comment in: *J Clin Oncol*. 2006 Jun 1;24(16):2414-6.

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Morgan MA, Reuter CW. Molecularly targeted therapies in myelodysplastic syndromes and acute myeloid leukemias. *Ann Hematol*. 2006 Mar;85(3):139-63.

Mesa R. A. Tipifarnib: farnesyl transferase inhibition at a crossroads. *Expert Rev Anticancer Ther*. 2006 Mar;6(3):313-9.

Maier SK, Hammond JM. Role of lenalidomide in the treatment of multiple myeloma and myelodysplastic syndrome. *Ann Pharmacother*. 2006 Feb;40(2):286-9.

Greenberg PL. Myelodysplastic syndromes: iron overload consequences and current chelating therapies. *J Natl Compr Canc Netw*. 2006 Jan;4(1):91-6.

Gore SD. Six (or more) drugs in search of a mechanism: DNA methyltransferase and histone deacetylase inhibitors in the treatment of myelodysplastic syndromes. *J Natl Compr Canc Netw*. 2006 Jan;4(1):83-90.

Naing A, Sokol L, List AF. Developmental therapeutics for myelodysplastic syndromes. *J Natl Compr Canc Netw*. 2006 Jan;4(1):78-82.

Steensma DP, Bennett JM. The myelodysplastic syndromes: diagnosis and treatment. *Mayo Clin Proc*. 2006 Jan;81(1):104-30.

Tehranchi R. Impact of growth factors in the regulation of apoptosis in low-risk myelodysplastic syndromes. *Med Oncol*. 2006;23(1):37-49.

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PNH

Okamoto M, Shichishima T, Noji H, et al. High frequency of several PIG-A mutations in patients with aplastic anemia and myelodysplastic syndrome. *Leukemia*. 2006 Apr;20(4):627-34.

Araten DJ, Luzzatto L. The mutation rate in PIG-A is normal in patients with Paroxysmal Nocturnal Hemoglobinuria (PNH). *Blood*. 2006 Mar 16; [E-Pub ahead of print].

Hill A, Ridley SH, Esser D., et al. Protection of erythrocytes from human complement-mediated lysis by membrane-targeted recombinant soluble CD59: a new approach to PNH therapy. *Blood*. 2006 Mar 1;107(5):2131-7.

Iron Overload

Borgna-Pignatti C, et al. Cardiac morbidity and mortality in deferoxamine- or deferiprone-treated patients with thalassemia major. *Blood*. 2006 107:3733-3737.

Cappellini MD, et al. A phase 3 study of deferasirox (ICL670), a once-daily oral iron chelator, in patients with beta-thalassemia. *Blood*. 2006 May, 107(9):3455-3462.

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Pennell DJ, et al. Randomized controlled trial of deferiprone or deferoxamine in beta-thalassemia major patients with asymptomatic myocardial siderosis. *Blood*. 2006 107:3738-3744. ♦

Our sincere gratitude to the Herman Goldman Foundation of New York City for sponsoring this newsletter.

Attention Medical Researchers

The AA&MDSIF is accepting applications for our Established and New Researcher Awards. We are interested in funding medical research studies relating ONLY to the prevention and treatment of bone marrow disease – aplastic anemia, Myelodysplastic syndromes, and paroxysmal nocturnal hemoglobinuria. Application forms can be downloaded: www.aamds.org

Combined Federal Campaign

Please encourage your co-workers to donate to the AA&MDSIF – Agency 0816 – through your federal place of employment. Remind them of our low administrative costs and ask them to compare our efficiency with other charities. Thank you!

Iron Overload Fund

The Health Well Foundation has launched the Iron Overload fund to provide deductible coinsurance and co-payment assistance for the treatment of iron overload because of blood transfusion. Patients and providers can obtain applications from (800) 675-8416 www.healthwellfoundation.org

Nationwide Study of MDS

Slone Epidemiology Center (SEC) at Boston University recently opened a nationwide registry of myelodysplastic syndromes (MDS). This novel study, Patient Registries at Slone: MDS, will research the impact of MDS and treatments received, if any, on patients' physical, emotional, social, and economic well-being. MDS patients are eligible to participate in this registry if they have been diagnosed within the past three months and are residents of the United States; eligibility is not related to the type of treatment. To learn more or to join the registry, visit www.aamds.org and click Patient Clinical Trials or call SEC directly at 800.231.3769. Physicians can also contact SEC to request enrollment packets to offer to patients.



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