

THE MDS FOUNDATION, INC. (MDSF) IS AN INTERNATIONAL ORGANIZATION DEVOTED TO THE SUPPORT AND EDUCATION OF PATIENTS AND HEALTHCARE PROVIDERS WITH INNOVATIVE RESEARCH IN THE FIELDS OF MDS AND RELATED MYELOID NEOPLASMS IN ORDER TO ACCELERATE PROGRESS LEADING TO THE CONTROL AND CURE OF THESE DISEASES. BY BUILDING AN INTERNATIONAL COMMUNITY OF PHYSICIANS, RESEARCHERS, AND PATIENTS, WE WILL MAKE POTENTIALLY CURATIVE THERAPIES AVAILABLE FOR ALL PATIENTS WITH MDS.

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TOGETHER, WE ARE A COMMUNITY RESOURCE OF HOPE FOR THOSE LIVING WITH MDS

2016 ANNUAL REPORT



Patient advocacy
and education

Professional
education

International
working groups

Research

MISSION STATEMENT

The MDS Foundation, Inc. (MDSF) is an international organization devoted to the support and education of patients and healthcare providers with innovative research in the fields of MDS and related myeloid neoplasms in order to accelerate progress leading to the control and cure of these diseases.

VISION STATEMENT

By building an international community of physicians, nurses, researchers, and patients, we will make potentially curative therapies available for all patients with MDS.

WHAT IS MDS?

Myelodysplastic Syndromes (MDS) are a group of diverse bone marrow disorders in which the bone marrow does not produce enough healthy blood cells. MDS is often referred to as a “bone marrow failure disorder”. MDS is primarily a disease of the elderly (most patients are older than age 65), but MDS can affect younger patients as well. To help you better understand MDS, it might be helpful to first consider some basics about bone marrow and blood. The bone marrow functions as a factory that manufactures three kinds of blood cells: red blood cells, white blood cells, and platelets. Healthy bone marrow produces immature blood cells — called stem cells, progenitor cells, or blasts — that normally develop into mature, fully functional red blood cells, white blood cells, and platelets. In MDS, these stem cells may not mature and may accumulate in the bone marrow or they may have a shortened life span, resulting in fewer than normal mature blood cells in the circulation.

**MORE THAN 500,000 PEOPLE SPECIFICALLY VISITED OUR
WHAT IS MDS? PAGE ON OUR WEBSITE IN 2016**

Low blood cell counts, referred to as cytopenias, are a hallmark feature of MDS and are responsible for some of the symptoms that MDS patients experience — anemia, infection, spontaneous bleeding, or easy bruising. Anemia (low red blood cell counts), neutropenia (low white blood cell counts), and thrombocytopenia (low platelet counts) are the major types of blood cell cytopenias, and are discussed below. In addition to reduced numbers of blood cells, the mature blood cells circulating in the blood may not function properly because of dysplasia. The formal definition of dysplasia is the abnormal shape and appearance, or morphology, of a cell. The prefix myelo- is from the Greek (*myelos*), meaning marrow; so myelodysplasia refers to the abnormal shape and appearance — or morphology — of the mature blood cells. Syndromes comes from the Greek (*dramein*), meaning to run, and with the prefix syn, means a set of symptoms that occur together.

Failure of the bone marrow to produce mature healthy cells is a gradual process, and therefore MDS is not necessarily a terminal disease. Some patients do succumb to the direct effects of the disease: reduced blood cell and/or reduced platelet counts may be accompanied by the loss of the body’s ability to fight infections and control bleeding. In addition, for roughly 30% of the patients diagnosed with MDS, this type of bone marrow failure syndrome will progress to acute myeloid leukemia (AML).

www.mds-foundation.org

**IN 2016 ALONE, OUR FOUNDATION HAD OVER 1 MILLION
WEBSITE HITS TO BETTER UNDERSTAND MDS**

FROM OUR CHAIRMAN

More than 20 years ago, the MDS Foundation, Inc. was established by an international group of physicians and researchers to provide an ongoing exchange of information relating to MDS. Until the Foundation was created, no formal organization had been devoted solely to MDS. Due to a persistent rise in awareness and diagnosis of MDS, as well as continued growth in the research efforts surrounding MDS, the need for our Foundation has only increased over time. Each year we continue to expand our reach worldwide to meet the many growing needs of the patients and families affected by myelodysplastic syndromes, as well as the healthcare professionals who dedicate their lives to caring for these patients, and researching treatment options and a cure for this disease.

WE UNDERSTAND WHAT IS IMPORTANT...

- For the newly diagnosed patient, offering a balance of education and gentle support, coupled with empowering each patient and caregiver with the tools to self-manage their disease and seek the best options for treatment.
- For our healthcare professional partners, offering educational programs that include the latest updates in MDS research, direct access to international working groups dedicated to the study of MDS and MPNs, and, when possible, the funds needed for this research.
- For our industry partners, the sharing of information related to treatments and clinical studies, while also working to incorporate the much needed patient voice into these clinical studies.

Overall, creating an environment where patients, families, and professionals effectively work together towards the common goals of better treatment options, improved quality of life, and eventually a cure for MDS.

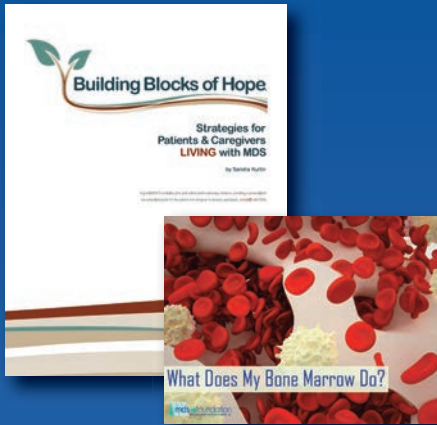
In our 2016 report, you'll see the work of a dedicated team committed to improving the lives of patients LIVING with MDS. Thanks to the tireless work of so many, the Foundation's efforts keep growing stronger. Thousands worldwide depend on the MDS Foundation for information, education and empowerment. We will continue to lead the way for the MDS community for many years to come.

Stephen Nimer, MD
Chair, MDS Foundation, Inc.



The MDS Foundation was established by an international group of physicians and researchers to provide an ongoing exchange of information relating to MDS.





"I simply wanted to say, after reading your main page, that it was extremely well written and informative. It was direct without being alarmist, seemed quite fair in not exploiting data or attempting to use incomplete or partial research to further your cause (therefore making what you do say much more trustworthy), and explained in very accessible terms everything one might want to know on a first investigation. Well done!"

Bill P.

"Each time I attend an MDS Foundation Patient Forum, I learn a little bit more about MDS. The presentations are always great."

Scott M.



PATIENT ADVOCACY

The first focus of the Foundation is patient advocacy, support and education. We provide extensive resources to patients and their families about MDS treatment options, upcoming clinical trials, and recent research so that they can make educated decisions about treatment and how to approach the disease. One such program is our *Building Blocks of Hope*® Patient and Caregiver Resource, which is an extensive print and online patient advocacy initiative that provides a personalized education program for the patient and caregiver to prepare, participate and LIVE with MDS.

MORE THAN 25,000 PEOPLE VIEWED THIS RESOURCE ON OUR WEBSITE IN 2016

We also provide various printed and electronic patient resources and handbooks that are available in multiple languages. In addition to the education component, the MDS Foundation develops patient support groups, hosts Quality-Of-Life Patient and Family Forums, and provides access to a full-time Patient Liaison who is available to advise and refer patients to the appropriate resources, studies, and/or specialists.

1,500 PRINTED INFORMATION PACKETS WERE DISTRIBUTED FREE OF CHARGE IN 2016

Every day approximately 238 people worldwide are diagnosed with MDS.

MDS CENTERS OF EXCELLENCE

As part of our patient referral system, we currently recognize **174** MDS Centers of Excellence worldwide. These treatment centers undergo careful review and must meet the following criteria:

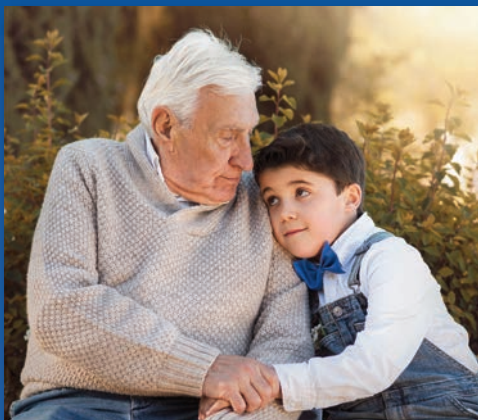
- An established university (or equivalent) program
- Recognized morphologic expertise in MDS
- Available cytogenetics and/or molecular genetics
- Ongoing research, including Institutional Review Board-approved clinical trials
- Documentation of peer-reviewed publications in the field

PROFESSIONAL EDUCATION

In an effort to advance medical research and improve the quality of healthcare for MDS patients, we disseminate information to professionals about new treatment options and facilitate an informational exchange between healthcare providers. To do this, we host an MDS symposium at the annual American Society of Hematology (ASH) Congress Meeting, which allows us to directly educate more than 600 hematologists, with additional access to 20,000 hematologists throughout the full congress meeting. We also hold a **BIENNIAL MDS INTERNATIONAL SYMPOSIUM**, with the 2017 symposium to be held in Valencia, Spain. For this event, we expect to host approximately 1,200 delegates and hold three workshops dedicated to specific MDS-related research developments, 10–12 plenary scientific sessions, which consist of abstract presentations, roundtables and debates, as well as an abstract poster viewing. We are also in the process of organizing a Corporate Satellite Symposia option, pharmacists session, and medical pipeline session for the 2017 Symposium. In addition to these programs, the MDS Foundation also maintains an online **CLINICAL TOOLBOX** resource for healthcare providers, and provides educational support for investigators. This clinical toolbox includes a **LEARNING MANAGEMENT SYSTEM** where professionals can earn continuing education credits.



In response to the needs expressed by patients, families, and healthcare professionals, we have established patient advocacy programs, professional education initiatives, and funded research efforts



"My father-in-law was just diagnosed with MDS. I came across your website and I am thrilled with the wealth of information I have gathered already. I believe these books will be a wonderful source of guidance. Thank you!"

Celena O.

"Thank you so much for all of your kind words of encouragement and all of the helpful information you have sent. This journey has a bright light for me and with people like you, I know it will be alright."

Rachel M.



INTERNATIONAL WORKING GROUPS AND GENERAL RESEARCH

The Foundation oversees three working groups dedicated to specific MDS-related topics:

INTERNATIONAL WORKING GROUPS



INTERNATIONAL WORKING GROUP FOR THE PROGNOSIS OF MDS (IWG-PM)

Responsible for continued revisions to the International Prognostic Scoring System (IPSS) for MDS.



MDS/MPN INTERNATIONAL WORKING GROUP (MDS/MPN IWG)

Developed to foster collaboration among translational scientists in the area of myeloid malignancy to better define, risk stratify, and treat patients with overlap syndrome.



INTERNATIONAL NURSE LEADERSHIP BOARD (NLB)

Composed of more than 30 members worldwide, and includes 3 subcommittees: *Professional Education*, *Patient Education*, and *Quality of Life*.

RESEARCH GRANTS

Over the last five years, **WE HAVE AWARDED MORE THAN \$300,000 IN GRANTS** through our Young Investigator Grant program. These funds have helped to make great strides in research into areas such as aberrant mRNA splicing induced by ZRSR2 mutation in the pathogenesis of myelodysplastic syndromes, unraveling the role of alternative splicing in normal and MDS hematopoietic stem and progenitor cells, HIF-1 α as a central pathobiologic mediator of myelodysplastic syndromes, and targeting TP53 gene mutations in myelodysplastic syndromes through functional reconstitution and immune activation. Providing the resources for these research projects directly improves the quality of life of MDS patients and their caregivers.

The Foundation also assists our industry partners through the clinical trial process by raising awareness of these vital trials, and incorporating the patient voice into these trials in an effort to increase enrollment of MDS patients. These research related efforts each year bring us closer to a cure for MDS.

PATIENT STORY

TALKING WITH OTHERS WITH RARE DISEASES EMPOWERS PATIENTS

MDS patient Ray Malles explains why he takes every opportunity to make his voice heard.

Being diagnosed with a rare disease like myelodysplastic syndromes (MDS) is scary, especially if you've never even heard of the disease. Sharing your experience with others who've been in a similar situation can be empowering. MDS patient Ray Malles, who's lived with MDS for nearly a decade, shares his thoughts on why it's important for patients to swap stories.



HOW LONG AGO WERE YOU DIAGNOSED WITH MDS?

I was diagnosed November 2006, when I was 76. We spent our winters in Florida, and my wife and I were told by our doctor to see a hematologist after some tests. When we approached the building, we saw that it said "Florida Cancer Institute" and just looked at each other. When the hematologist told me that I had MDS, I said, "What's that?"

HOW DID YOU REACT?

I'm the kind of person who doesn't just do what others tell me. I wanted to know everything about my disease. I rolled up my sleeves, read articles and contacted the MDS Foundation. Somewhere around 2011, my doctors told me that my numbers were dropping and recommended a blood transfusion. I had learned enough about the complications associated with blood transfusions and told them that I didn't want it. So we explored other treatment options that have been working just fine for me so far.

IT SEEMS LIKE BEING AN ACTIVE PARTICIPANT IN YOUR TREATMENT IS IMPORTANT TO YOU. DO YOU SEE THAT IN OTHER MDS PATIENTS AS WELL?

There are all kinds of people that make up this world of ours. I don't think that patients should feel like they are hostages. They shouldn't put their heads in the sand and never question anything. I want to know what my disease is and what the side effects of my treatment are.

ARE THERE OTHER WAYS THAT YOU HAVE MADE YOUR VOICE HEARD?

I've produced a series of YouTube videos to educate people on MDS and have attended several MDS patient forums. I've become a very big patient advocate of those forums. After my diagnosis, my daughter discovered that one was taking place in Philadelphia, so all three of us—my daughter, my wife and I—participated. I've learned a lot from talking with other patients and have given presentations in my community. Patient-to-patient communication is very important for MDS patients, or any patient with a rare disease.

I want to know what my disease is and what the side effects of my treatment are.

Patient-to-patient communication is very important for MDS patients, or any patient with a rare disease.



"I have problems keeping up with MDS research and new MDS medications available. Your MDS website and newsletter helps the most with these issues. Please keep up the good work you do."

William H.



WHEN YOU TALK WITH OTHER PATIENTS, WHAT DO YOU HOPE THEY TAKE AWAY?

Doctors sometimes throw these big words around, and that can be intimidating. Hopefully, I'm encouraging them to be an active participant in their treatment. Let me give you an example. My daughter works for a medical practice in North Carolina and has been in contact with two other people over the past 8 years with MDS. She reached out to them and asked if they would like to speak with me. I answered their questions from my perspective, and they were very appreciative.

SINCE THE ORPHAN DRUG ACT OF 1983, THE U.S. FOOD AND DRUG ADMINISTRATION HAS APPROVED OVER 500 TREATMENTS FOR RARE DISEASES. WHAT ADVICE WOULD YOU GIVE TO THE FDA WITH REGARD TO NEW TREATMENTS FOR RARE DISEASES?

I once attended a session about the number of steps involved in the clinical trial and drug approval process. I know that the objective is to protect patient health and make sure the therapies are safe for patients. But 10 years to move a treatment from concept to market? There are a lot of patients who can benefit during that time. The FDA should do everything that they can to remove any unnecessary barriers to getting therapies approved as quickly as possible.

MOVING FORWARD

In addition to our current patient and professional programming, the Foundation will be moving forward in some new and exciting ways...

- **PEDIATRIC PROGRAMMING** including online and printed resources specific to pediatric MDS patients and families, a Pediatric MDS Centers of Excellence program, and a pediatric patient and family advisory board.
- **MPN RESOURCES** — the development of MPN online and printed educational resources, including an MPN *Building Blocks of Hope*; and an MPN mHealth application — *MPN Manager* that includes tools to improve health and illness self-management, self-efficacy, health literacy, technology literacy, health communication, shared decision-making and empowerment.
- The continuation of our **BIENNIAL MDS-SPECIFIC MEETINGS** devoted to healthcare professionals — the 15th International Symposium on Myelodysplastic Syndromes in Copenhagen, Denmark, May 8–11, 2019.
- An increase in overall development efforts with the addition of a **DIRECTOR OF DEVELOPMENT**. These efforts will result in a broadening of our funding sources, allowing the Foundation to expand and provide even more programs for the patients and professionals we serve.



MDS FOUNDATION MEMBERSHIP

WHAT ARE MDS MEMBERSHIP BENEFITS?

- Being part of the solution to change MDS outcomes. Membership fees help support global physician and patient educational initiatives, and help to empower patients with courage and hope.
- Two printed issues of *The MDS News*, which includes the latest on MDS as well as exceptional patient and caregiver stories.
- Regular updates on the status of our Global Centers of Excellence and their patient events that encourage collaboration.
- Information on the latest clinical trials to potentially share or participate in.
- Access to MDS awareness materials to share with family and friends.
- Opportunities to participate in or host support group events with your friends and community.

MDS PATIENT MEMBERSHIP OPTIONS

- \$35** *Community Membership* (includes benefits listed above)
- \$70** *Sharing Hope Membership* (includes benefits listed above as well as a membership scholarship for a patient or caregiver in need)
- \$250** *Changing the Future of MDS Membership* (includes benefits listed above as well as additional support for the MDS Foundation as we work together to change the future of MDS) Member names are listed on the MDSF website.

MDS PROFESSIONAL MEMBERSHIP OPTIONS

- \$35** *Community Professional Membership* (includes discounted registration rates at MDSF meetings, discounted subscription rates to Leukemia Research, as well as access to MDSF resources for distribution to your patients)
- \$250** *Changing the Future of MDS Professional Membership* (includes discounted registration rates at MDSF meetings, discounted subscription rates to Leukemia Research, access to MDSF resources for distribution to your patients, as well as the opportunity to present at MDSF patient events in your region. In addition, \$50 of your membership will help support a Professional outside of the United States that represents a CoE in financial need. Member names are listed on the MDSF website.



TO BECOME A MEMBER VISIT:

<https://www.mds-foundation.org/membership>

HOW DOES MEMBERSHIP HELP?

- Supports over 1,000 educational packets to families and caregivers free of charge annually, to help navigate through their MDS diagnosis.
- Helps our Patient Liaison respond to over 1,300 on-line requests annually.
- Supports over 170 Centers of Excellence worldwide. We believe this is imperative as these centers serve as our patient referral base, and this partnership helps the MDS community collaborate and engage in innovative practices in the diagnosis and care of MDS patients.
- Helps to distribute over 8,000 translated pieces of MDS materials annually.
- Enables MDSF to support approximately 250 professionals collaborating through International Working Groups — with researchers in 37 countries, and on 6 of the 7 continents.
- Helps to educate patients, caregivers and professionals at live events. This year MDSF will host our International Symposia in Valencia, Spain. We anticipate 1,200 professionals in attendance. We will also host 11 live patient events.
- Helps the MDS Foundation develop the growth of our *Pediatric Centers of Excellence* program to support children and their families who are living with MDS.



"My father was diagnosed with MDS in Oct 2016 and this is the best site I have found online. I would love to provide him with these books of information about MDS. Thank you."

Rebecca V.

"Thank you for making these books available. They're the most accessible I've found so far."

Maya T.

"Thank you for providing a booklet that addresses many of my questions and provides resources to seek answers to other questions."

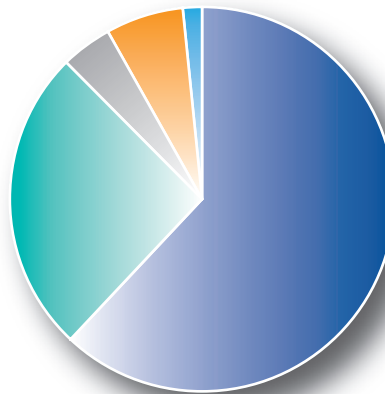
Lindsay G.



THE MDS FOUNDATION IS A
PROUD FOUNDING MEMBER
OF THE MDS ALLIANCE!

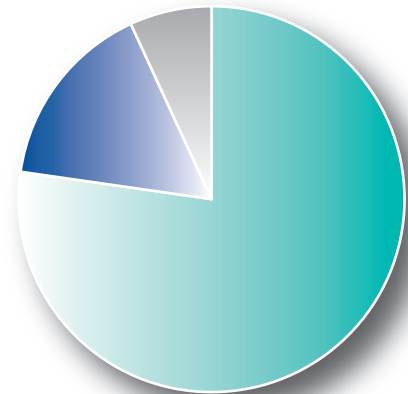
FINANCIALS

2016 REVENUE



- Industry Grants (62%)
- Donations (26%)
- Membership Dues (6%)
- Program Service (4%)
- In Kind Donations (2%)

2016 EXPENSES



- Program Services (77%)
- Management & General (16%)
- Marketing and Fundraising (7%)

INDUSTRY PARTNERS

Thank you to our industry partners for their continued support of the MDS Foundation. Our dedicated work to aid and educate patients and professionals is made possible through these valuable partnerships!

Amgen Inc.

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Incyte Corporation

Janssen Pharmaceuticals Inc.

Novartis Pharmaceuticals

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Thank you to
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