

the myelodysplastic syndromes foundation, inc.

2017 ANNUAL REPORT

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



Patient
advocacy
and education

Professional
education

International
working
groups

Research

MISSION STATEMENT

The MDS Foundation, Inc. is an international non-profit advocacy organization whose mission is to support and educate patients and healthcare providers with innovative research into the fields of MDS, Acute Myeloid Leukemia (AML) and related myeloid neoplasms in order to accelerate progress leading to the diagnosis, control and cure of these diseases.



WHAT IS MDS?

The myelodysplastic syndromes (MDS) are a group of bone marrow failure disorders. The bone marrow is the factory for the production of blood cells including red blood cells (energy), white blood cells (immune system), and platelets (clotting). In MDS, the bone marrow is damaged and fails to produce enough blood cells (cytopenias).

MDS is considered a rare disease, with relatively low incidence rates worldwide. However, the incidence may be increasing as the population ages and more individuals are exposed to toxic chemicals and/or prior chemotherapy treatments.

IN 2017 ALONE, OUR FOUNDATION HAD OVER 1 MILLION WEBSITE HITS TO BETTER UNDERSTAND MDS

www.mds-foundation.org

FROM OUR CHAIRMAN

Dear Friends and Colleagues,

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. As we continue our mission to educate patients and professionals worldwide, we have expanded our Medical and Scientific Advisory Board to include physicians from Israel and Spain.

NEW FOCUS

In 2017, we recognized a need to expand our focus to include education and research into related myeloproliferative neoplasms (MPN), the so called overlap syndromes, especially given the similarities to MDS, in the clinical presentation of these disorders and the mutations that accompany them. Our new MDS/MPN patient and caregiver resource will be completed in 2018 and we now support the efforts of a dedicated working group of global MPN professionals (MDS/MPN IWG) with plans to launch the first MDS/MPN IWG study: *A Novel Therapy Combination in Untreated MDS/MPN and Relapsed/Refractory Overlap Syndromes (ABNL-MARRO)*. ABNL-MARRO is an international basket study designed to allow new compounds and combinations of therapy to be introduced easily among MDS/MPN IWG clinical sites which see MDS/MPN patients, study the biology and pathophysiology of the diseases, and have multilateral expertise in this area. ABNL MARRO-001 is the first MDS/MPN IWG study and is planned to begin in mid-2018 with first patient in scheduled for March 1, 2019.

In our 2017 report, you'll see the work of a dedicated team committed to improving the lives of patients LIVING with MDS and MPNs. 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. The research into MDS and MPNs has grown, with more professionals dedicated to these diseases than ever before. We are beginning to learn more about the biology surrounding myeloid diseases and due to these discoveries, **the future looks bright for patients and families battling these diseases.**

Given the dedication and expertise of the ever increasing members of our community, we are proud to continue to lead the way for this community for years to come.

Stephen D. Nimer, MD
Chair, MDS Foundation, Inc.



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PATIENT RESOURCES

The primary 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 patients can make educated decisions about treatment and take a more active approach to their care. One such program is our *Building Blocks of Hope*[®] – *Strategies for Patient and Caregivers LIVING with MDS*, which is an extensive print and online patient advocacy initiative that provides a personalized education program.

MORE THAN 25,000 PEOPLE VIEWED THE BUILDING BLOCKS OF HOPE RESOURCE ON OUR WEBSITE IN 2017

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 live 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.

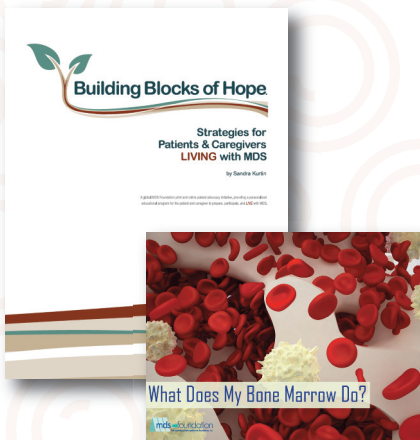
1,500 PATIENT AND CAREGIVER INFORMATION PACKETS WERE DISTRIBUTED FREE OF CHARGE IN 2017

Every day approximately 238 people worldwide are diagnosed with MDS.

MDS CENTERS OF EXCELLENCE

As part of our patient referral system, we currently recognize **181** 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



MDS MANAGER

MDS Manager™ is a newly developed mobile app for smartphones and tablets!

This app includes a variety of features to assist patients and their caregivers living with MDS to more effectively manage their care, improve communication with providers, and track their symptoms and response to treatment.

"Your app has been extremely helpful to me and makes it so much easier than having to cart around all that paper."

Nancy R.

PROFESSIONAL EDUCATION

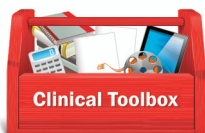
In an effort to advance medical research and improve the quality of healthcare for MDS patients, we disseminate information to professionals about new treatment options and facilitate an informational exchange between healthcare providers.



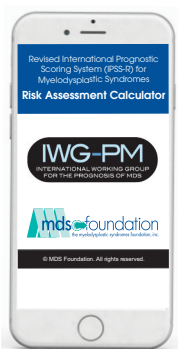
We host an **MDS BREAKFAST SYMPOSIUM AT THE ANNUAL AMERICAN SOCIETY OF HEMATOLOGY (ASH) Congress Meeting**, which allows us to directly educate more than **600 hematologists**, with additional access to 20,000 hematologists throughout the full congress meeting.



We hold a **BIENNIAL MDS INTERNATIONAL SYMPOSIUM**, with the 2017 symposium held in Valencia, Spain. For these events, we 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. In Valencia, we organized a Corporate Satellite Symposia option, pharmacists session, and medical pipeline session.



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 MEDICAL EDUCATION CREDITS**.



IPSS-R CALCULATORS

Our Online Basic Calculator

The Basic calculator tool includes clinical features of marrow blasts, cytogenetics, depth of cytopenias and age.

Our Online Advanced Calculator

The Advanced calculator tool includes the above features plus the additive differentiate features for patient survival of performance status, serum ferritin, LDH, beta-2 micro globulin and marrow fibrosis.



12-20K

new cases of MDS are reported every year in the U.S., with an average of **33-55** people diagnosed in the U.S. every day.

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



60-170K
people

are estimated to live with MDS in the U.S. with an estimated **87,000** new cases each year worldwide.^{1,2}

"Encouraged to come across your website. I am feeling overwhelmed by all the technical, medical sites I have been researching. Your introduction video is a comfort for 'regular' people, who have limited knowledge of this syndrome. "

Rebecca 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 PROGNOSIS IN MDS (IWG-PM)

The objective of this group is to continue to refine the currently accepted and utilized prognostic scoring systems based on the current research discoveries. (Responsible for continued revisions to the International Prognostic Scoring System (IPSS) for MDS.)



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

The overarching goal of this group is to identify key knowledge gaps in the area of MDS/MPNs (Myeloproliferative Neoplasms) and facilitate international, collaborative, translational science geared to rapidly improve our understanding of these neoplasms. (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

From 2012–2017, **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

It was a call she would never forget: a diagnosis she could hardly pronounce, much less understand. Myelodysplastic Syndromes. MDS. How could this be happening? What did it mean for her? And what would it mean for her seven-year-old daughter?

Julia's diagnosis left her mind reeling and her heart breaking. After a quick online search, she found the MDS Foundation, Inc. Among all of the information it offered her and her family, one piece proved especially crucial: a list of MDS Centers of Excellence. This was her path to hope — and a plan.



MDS FOUNDATION
CENTER OF EXCELLENCE
2017-18 DESIGNATED

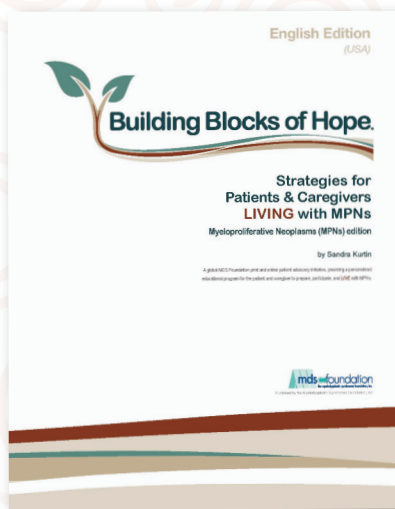
Within three days, Julia had connected with one of the country's leading MDS clinical/research teams. As her local hematologist told her, "those doctors are five years ahead of the rest of us." Under her specialist's care, Julia found a new treatment plan based on the latest research and practices. Nearly four years after that traumatic

call, Julia's blood counts are stable and she is busy working fulltime, being a hockey and Irish dance mom, chaperoning 5th grade field trips, and feeling grateful everyday.

**JULIA IS A VALUED ADVISOR
TO THE MDS FOUNDATION
DEVELOPMENT BOARD**



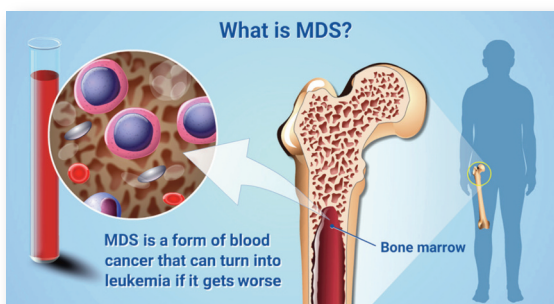
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MOVING FORWARD

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

- **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.
- **YOU AND MDS and YOU AND AML – An Animated Patient’s Guide to Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia (AML)**



Easy-to-understand animations with audio narration, expert video explanations, patient interviews, illustrated slide shows, and educational downloads. Viewers are invited to provide feedback to help direct future content. We are

excited about this new online community resource to improve quality of life and health outcomes.

- First-Ever **MDS AWARENESS WALK** to raise awareness of myelodysplastic syndromes (MDS).

MDS FOUNDATION MOBILE APP



The MDSF App is a newly developed mobile app for smartphones and tablets!

This app provides patients, caregivers, and healthcare providers with quick access to the important services that the MDS Foundation provides. These services include our worldwide Centers of Excellence, upcoming Patient Forums and Events, as well as our numerous online resources.



- 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.



MDS FOUNDATION MEMBERSHIP

BENEFITS OF MEMBERSHIP

- You are part of the solution to change MDS outcomes. Your membership fee helps support global physician and patient educational initiatives, and helps to empower patients with courage and hope.
- Updates on the status of our Global Centers of Excellence and their live patient and family forum events that allow for more rapid dissemination of new research and treatment developments.
- 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.
- Receive two printed issues of *The MDS News*, which includes the latest on MDS research as well as inspiring patient and caregiver stories.

YOUR MEMBERSHIP MAKES A DIFFERENCE

\$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.



"She asked if I would like for her to make an appointment. We had an appointment WITHIN A WEEK and were treated royally. That is some seriously appreciated clout. Now anyone out there experiencing MDS in your family or with friends I tell from experience there is ONLY ONE KIND of doctor you should be seeing: A DOCTOR RECOMMENDED BY THE MDS FOUNDATION"

The Fournier Family

Tim Fournier, MDS Patient, 79 years old,
3 children, 8 grandchildren

TO BECOME A MEMBER VISIT:

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



"It took a long time to wrap our heads around a disease without a cure when my husband felt just fine – The MDS Foundation gave me the information I so badly needed to be a good caregiver. The MDS Foundation was there when we needed it desperately."

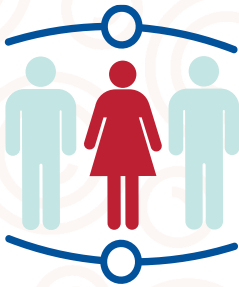
The Cook Family

Janice Cook, MDS Caregiver,
70 years old, 2 children, 1 grandchild
("Grandpa's best medicine")

"When I was diagnosed with MDS in 2008, the MDS Foundation became my primary source of accurate, comprehensive and understandable information about this complex and challenging bone marrow disease.

I donate to the MDS Foundation because it's an unparalleled resource for patients, caregivers, treatment providers and researchers. Additionally, I donate because of the wonderful, caring professional staff."

MDS patient, 68 years old



1 OUT OF 3

MDS patients (or 30%)
progress to acute
myeloid leukemia (AML).

*"I am writing to say a huge
THANK YOU for the booklet.
It is just what I needed, very
upbeat and a welcome answer to
my many questions."*

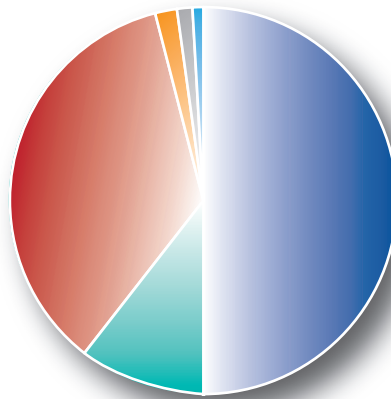
Jan S.



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

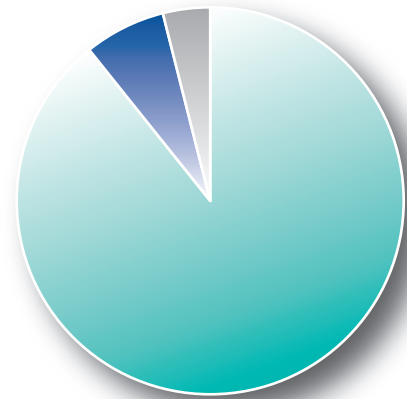
FINANCIALS

2017 REVENUE



- Industry Grants (50%)
- Special Events/Symposia (36%)
- Donations (10%)
- Membership Dues (2%)
- Program Service (1%)
- In Kind Donations (1%)

2017 EXPENSES



- Program Services (89%)
- Management & General (7%)
- Fundraising (4%)

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!

- Agios
- Astex Pharmaceuticals
- Celgene Corporation
- Daiichi Sankyo
- Incyte Corporation
- Janssen Pharmaceuticals Inc.
- Jazz Pharmaceuticals
- Novartis Pharmaceuticals
- Onconova Therapeutics Inc.
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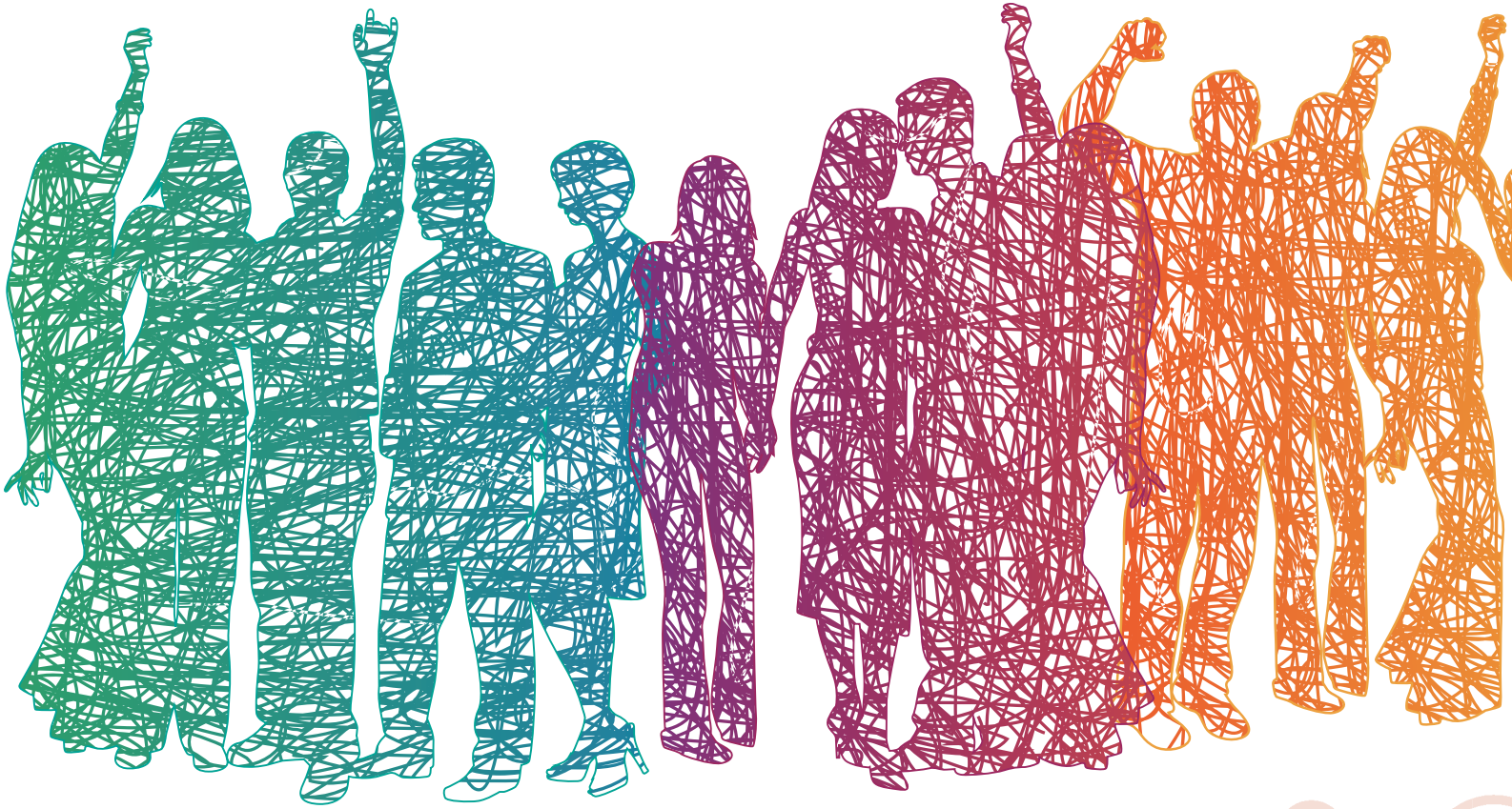
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www.mds-foundation.org

The MDS Foundation is a publicly supported organization,
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