

#### TABLE OF CONTENTS

A Message from Our Directors	(
The Impact of Your Dollar	4
Faces of MDS: Fran Boyle	<u>{</u>
Strategic Goals	
2020 At A Glance	7-8
Ways You Made An Impact	ç
MDS Awareness Walks	10
New Treatment Options for MDS	1
Faces of MDS: Nathaniel Montgomery	12
How You Can Get Involved	13
Financial Highlights	14-15
Our Supporters	1c
Our Team	

# WHAT IS



Myelodysplastic syndromes (MDS) are an often unrecognized, underdiagnosed rare group of bone marrow failure disorders, where the body no longer makes enough healthy, normal blood cells in the bone marrow. The disease is also known as a form of blood cancer.

#### A MESSAGE FROM OUR DIRECTORS

Tracey Iraca & Stephen Nimer, MD





To say that 2020 was a challenging year would be a gross understatement. The pandemic affected us all worldwide, but COVID 19's devastating effects hit our healthcare professionals and our patients especially hard. However, we're very proud of how we came together as a community. The MDS Foundation made the necessary changes to our programming to ensure that the patients, families, and healthcare professionals we serve continued to receive the same excellent level of education and support. Our live Patient and Family Forums were transitioned to online webinars serving an even larger and more global community; our annual American Society of Hematology (ASH) symposium for healthcare professionals saw a 30% increase in participation in a virtual setting; and COVID 19 did not stop us from spreading much needed awareness of MDS and the MDS Foundation through creative and interactive virtual run/walk events.

With exciting new research and advancements in the fields of MDS, AML and MPNs, we embarked on and completed a strategic planning process in 2020. We identified 5 key strategic imperatives to maximize the local and international impact of the MDS Foundation: Education & Patient Support, Research, Awareness, Partnership & Funding, and International Relations.

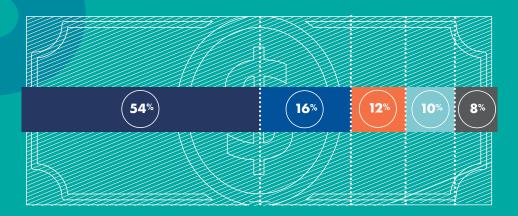
In 2021, we need to further identify and address the health disparities that exist in the treatment of hematologic malignancies. We're launching a new program forward on health disparities in blood disorders, beginning with ageism. We'll create an online platform where people diagnosed with these disorders can share their stories and ask questions. We believe that giving a voice to people with MDS, AML and MPNs will shed light on the many health disparities they face.

Thousands worldwide depend on the MDS Foundation for information, education, and empowerment. Given the dedication and strength of our ever-growing community, we will continue to advance important initiatives and lead the way towards a better life, with more hope for all we touch.

**Executive Director** 

Board of Directors, Chairman

# THE IMPACT OF YOUR DOLLAR



### (54%) Patient Advocacy

Support and resources for patients and caregivers:

- Patient and Caregiver Advocacy, Support and Education
- In person and online communities connecting patients, caregivers and others affected by MDS
- Regional patient forums
- Support and Educational Resources



### **Professional Education & Trainina**

Professional education initiatives for healthcare providers:

- International MDS Foundation Congress Events
- Regional MDS Foundation Symposia
- Annual American Society of Hematology Symposia
- Professional Learning Center with CME opportunities



# **Fundraising**

Events that focus on awareness of MDS and fundraising towards the mission of the MDS Foundation.



### **Management & General**

Representing general operating expenses for directing the affairs of the MDS Foundation:

- Customer relations management
- Finance
- Human resources
- Information technology services



#### Research

Research opportunities for investigators to initiate, continue or complete projects that focus on basic or clinical management into the causation, epidemiology, molecular biology, cytogenetics, morphology, prognosis and treatment of the myelodysplastic syndromes:

- Young Investigator Grants
- International Working Group for the Prognosis in MDS (IWG-PM)
- MDS/MPN International Working Group (MDS/MPN IWG)

3 | MDS FOUNDATION 2020 ANNUAL REPORT | 4









THE STORY OF FRAN BOYLE

My MDS Journey started in 2000 during a physical, when my doctor noticed my HGB was declining. After this occurred three years in a row, he referred me to a local Hematologist. I returned to the Hematologist yearly until he did a bone marrow biopsy in 2006. Results showed I had Refractory Anemia with Ringed Sideroblasts (RARS). My HGB was around 10-11. I was 56.

The Hematologist told me not to look it up online as I could have this for years without needing treatment. It was only after searching online that I learned I had a blood cancer and found the MDS foundation. I realized I needed to go to their Center of Excellence. I consulted with Dr. Porter at University of Penn where I had my second biopsy with the same result - RARS. Two of my brothers were eligible to get tested as a bone marrow donor. Neither of them were a match and I was not considered a good candidate for a bone marrow transplant at the time.

I then saw Dr. Emanuel Besa, whom I had met at an MDS Foundation seminar. He was conducting his own drug trial that had achieved positive results showing Accutane could delay the need for transfusions. I took Accutane for about a year, but began to need regular transfusions in 2009. Dr. Besa referred me to Dr. Erev Tubb at Crozer Hospital. Under

Dr. Tubb I tried Procrit and Aranesp without success. I started on Revlimid. I was required to have bone marrow biopsies before treatment began and after it failed. I was transfusion independent for 143 days before it stopped working. After a second attempt with no response I tried Vidaza in early 2011, which ultimately failed.

The nurses at Crozer were terrific, but I decided that practice was not for me. I consulted the MDS Foundation Message Board and was introduced to Bob Weinberg. Bob was on the board of directors at MDS Foundation and was diagnosed with MDS in his late 40's. He changed my life by recommending Dr. Cliff Pemberton. At my first appointment with Dr. Pemberton, my HGB was mid 6's and I was not doing well. He immediately ordered 2 units and I finally felt alive again. Eight years later I have many antibodies, but the blood bank always comes through!

At present, I am 70 years old and in a good place despite having MDS for 14 years. The hardest thing about MDS is the fear of the unknown and the worry it causes my husband and daughter. I try not to dwell on that, as long as I have the help and support of my family, doctor, and nurses I'm good!

# STRATEGIC GOALS

MAXIMIZE LOCAL AND INTERNATIONAL IMPACT OF MDS FOUNDATION.







#### **AWARENESS**

Ensure physicians and their networks are communicating valuable MDS Foundation resources to patients and their caregiving communities.

## RESEARCH

Support innovative research in MDS, and the related areas of MDS/MPN, clonal hematopoiesis, and secondary AML.

# **EDUCATION** & SUPPORT

Maintain, enhance, and evolve support and educational initiatives to solidify positioning as the global leader in MDSrelated education.



### **PARTNERSHIP & FUNDING**

Develop strong partnerships and maximize donations, support, and resources across patients and their caregiver communities, healthcare professionals and industry partners.

5 | MDS FOUNDATION 2020 ANNUAL REPORT | 6

# **AT A GLANCE**

MDS FOUNDATION HIGHLIGHTS

### **Education Features: 8 Webinars in 2020**

Live webinars provided uninterrupted access to information during the height of the pandemic. Speakers presented overviews on topics ranging from high-risk to low-risk MDS, stem cell transplant, and treatment options. Patients and caregivers engaged experts in meaningful discussion and learning during the series.

#### **Patient Concerns**

The pandemic led to 80% fewer cancer screenings, delayed treatments, and overburdened healthcare professionals. We responded to patient concerns by converting in-person support groups and spring walks to virtual format and providing 2,500 masks for the protection of patients and caregivers.

### **Published 'Out of Shape'**

In Out of Shape, MDS patient and advocate Bergit Korschan-Kuhle describes life after a diagnosis. The autobiography shares 15 years of patient experience, detailing the evolution of MDS information. Out of Shape is available at no-cost through our publication.

#### 2 New COEs Added

Expanded our Centers of Excellence to 197 healthcare facilities, internationally. Our centers are recognized for their expertise in MDS and contribution to on-going research.

### **Kicked Off Knowledge is Power**

Knowledge is Power, an on-demand platform which empowers patients and caregivers to know their score, know their subtype, and know their mutation in order to take an active role in decisions about treatment and advocate for options to prolong and improve the quality of their lives.

### **2nd Regional Symposium in Tel Aviv**

The 2nd Regional MDS symposium took place in Tel Aviv, despite early pandemic complications. The symposium combined educational sessions, emerging research topics, and discussions of real-world patient concerns.

Experts from around the world led a scientific program covering the genetics, epidemiology, and pathogenesis of the disease. Two days of social and cultural events at the scientific conference set the tone for future MDS symposiums.







**FOR MORE INFO VISIT MDS-FOUNDATION.ORG** 



# WAYS YOU MADE AN IMPACT IN 2020

YOUR SUPPORT ALLOWED US TO:

#### **Go Virtual**

Given the limitations due to the pandemic, we were able to swiftly move to virtual patient forum events, patient support groups, and provide robust resources online for patients with MDS, their caregivers, and those impacted by the disease.

#### Advance Clinical Trial Awareness

Establish a procedure for assisting the biotechnology and pharmaceutical industries with creating awareness for clinical trials in the fields of MDS, AML, and related myeloid neoplasms. With our active awareness campaign and resources, we have been able to successfully help patients toward our mutual goal of finding better treatment options and a cure.

#### Launch "Art Works: Visions of Life with MDS"

Create a gallery by members of the MDS community to help the world understand the challenges of living with MDS, while also celebrating personal stories of love, hope and strength.

#### **Grow Our Community**

Grow our social media channels to drive awareness of MDS and connect patients to their community and resources.

# Provide a Comprehensive Update to Building Blocks of Hope

Global print and online patient advocacy initiative, providing a personalized education program for patients and caregivers to prepare, participate, and live with MDS.

# 2020 MDS AWARENESS WALKS

The MDS Awareness Walks/Runs held in Nashville, New York City, Chicago and Boston helped to spread awareness and bring attention to this disease among the physician community as well as the general public.

The walks elevated the conversation around the unmet needs of those living with MDS by bringing together the MDS and rare disease community. New connections were created and committed to finding ways to improve the lives of MDS patients and those who care for them.

Momentum has been created for future MDS walks in more cities across the country!





BOSTON/ GLOBAL Awareness Walk



2ND ANNUAL
CHICAGO
Awareness Walk



1ST ANNUAL
NASHVILLE
Awareness Walk



NEW
YORK CITY
Awareness Walk

9 | MDS FOUNDATION

# NEW TREATMENT OPTIONS FOR MDS



After a long and frustrating 13-year pause, new treatment options are available for patients with myelodysplastic syndromes (MDS). Luspatercept-aamt was approved by the Food and Drug Administration (FDA) on April 4, 2020 for treatment of adult patients with very low to intermediate-risk myelodysplastic syndromes with ring sideroblasts or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis.¹ This can be used for the treatment of anemia in subtypes of MDS after an erythropoiesis stimulating agent failure and require 2 or more red blood cell units over 8 weeks.² Subsequent approval later this year was a fixed dose oral combination of decitabine and cedazuridine (Inqovi®) in July.³ During the COVID-19 pandemic, this oral treatment option was approved for treatment of adults with MDS subtypes of refractory anemia, refractory anemia with ringed sideroblasts, and refractory anemia with excess blasts with risk scores of intermediate-1, intermediate-2 and high-risk according to the International Prognostic Scoring System groups.⁴ Inqovi demonstrated similar pharmakinetics, pharmacodynamics and response rates to intravenous decitabine.

In September, magrolimab was granted breakthrough therapy designation4 for the treatment of newly diagnosed MDS in combination with azacytidine based on promising phase I/II data presented at European Hematology Association.<sup>6</sup>

This first in class monoclonal antibody blocking CD47 receptor which is overexpressed in MDS and AML known as the "do not eat me signal" which allows evasion of macrophages cell killing is being studied in patients with higher risk MDS. Another agent with encouraging results when used in combination with azacytidine is APR -246, a TP53 modulator restoring its wild type function, was selected by the FDA in April for fast-track orphan drug designation. It is being studied in higher-risk MDS patients, focused on individuals with the TP53 mutation.<sup>8</sup> Finally, imetelstat is in phase 2/3 studies and was also granted fast-track designation by the FDA for transfusion dependent patients with low or intermediate-1 MDS who do not have del (5q) and who are refractory or resistant to erythropoiesis stimulating agents.<sup>9</sup>

The future is brighter now for patients diagnosed with MDS, with new treatment options available and agents on the horizon!

**NEWS**WIRE

age 1

# Large International Study Pinpoints Impact of TP53 Mutations on Blood Cancer Severity

Having two mutated copies of the TP53 gene - as opposed to a single mutated copy - is associated with worse outcomes in myelodysplastic syndrome and acute myeloid leukemia

THE FACES OF







#### THE STORY OF NATHANIEL MONTGOMERY

My name is Nathaniel Montgomery. I am a cancer survivor and a fan of those who dedicate their lives to fighting cancer. I was diagnosed with Myelodysplastic Syndromes (MDS) in January of 2017. As a healthy and active African-American man who never smoked or drank alcohol I was stunned to learn that I had MDS.

My wife and I traveled coast-to coast seeking second and third opinions from MDS specialists, and assurance of a positive outcome. Each specialist we spoke with confirmed my diagnosis and politely explained the available treatment options. The most comforting aspect of my consultations were the acknowledgements of the medical advancements related to MDS. It was clear to me that I needed to identify a viable bone marrow donor.

I worked with the wonderful team at Memorial Sloan Kettering Cancer Center. Once the coronavirus spread throughout New York City, my wife and I decided that it was best to transfer to the Vanderbilt University Medical Center (VUMC) in Tennessee. The transplant team at VUMC welcomed me with open arms, providing information and support in navigating the transplant process. The team at VUMC notified me that a bone marrow donor had been identified, and we could

move forward with a transplant. I don't know if I was more nervous about the bone marrow transplant or relocating halfway across the country. Nevertheless, my wife and I made the move to Nashville, and I began chemotherapy. After undergoing chemotherapy, the donor's stem cells arrived, and the bone marrow transplant was performed.

#### The actions of one person not only changed my outlook on life but changed the outlook of those who depend on me.

My donor is a highlight in my story, but not everyone on my journey fared as well as I did. The loss of two very special friends was a stark reminder that there is more work to be done in the fight against cancer. I am motivated to share my journey because of the selflessness of my donor and the unfortunate loss of two special friends who were battling cancer this past year. It is my pledge to serve as a voice in the African-American community for those who are currently battling cancer and for those who might be faced with this terrible disease in the future. It is my intention to use this article as a plea to encourage individuals of color to consider becoming donors. I dedicate this article to my donor, my colleague's wife, and my former mentor and friend.



#### **Become a Member**

Become a Member of the MDS Foundation community. Get access to patient advocacy services and support the mission of improving the lives of patients with myelodysplastic syndromes.

Join online at mds-foundation.org.



### **Start a Support Group**

Support groups provide patients, caregivers and their family members with a network of individuals experiencing similar issues. We will provide you with a toolkit and materials to start your own support group.

### **Host a Fundraising Event**

You can create your own fundraiser event including golf tournaments, special dinner events, community runs or just about anything else you can dream up. If you want something that requires a little less effort, you can create your own virtual fundraiser on our Facebook page. Easy to do and is a great way to grow awareness for MDS with your social network.

**VISIT MDS-FOUNDATION.ORG** TO DISCOVER HOW YOU **CAN JOIN, DONATE AND VOLUNTEER.** 

#### **Attend a Foundation Educational Event**

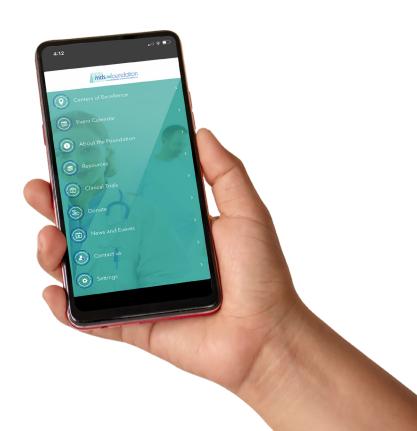
Learn about the most updated treatment options, clinical studies, referral opportunities to COE specialists, and updated information concerning MDS, acute myeloid leukemia (AML), and other related myeloid neoplasms (MPNs).

#### Submit your story to be shared with others

You will be able to share your experience and unique insights by submitting your story, which can provide comfort to those going through something similar. You'll likely find it therapeutic for yourself too.

### **Download the MDS Foundation Mobile App**

Have MDS information at your fingertips! The app provides patients, caregivers, and healthcare providers with guick 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. Available in the Google Play Store and iTunes.



#### FOR HEALTHCARE PROFESSIONALS:

#### Apply to be listed as a Center of Excellence in MDS

Would you like your treatment center to become part of the referral system for MDS patients and be designated as a Center of Excellence? Visit us online to find out how to qualify and apply.

#### Submit your research studies and publications in MDS to be shared with others

We are happy to share new developments and research with our audiences. Please contact the Foundation office to learn more.

#### **Apply for a Young Investigator Grant**

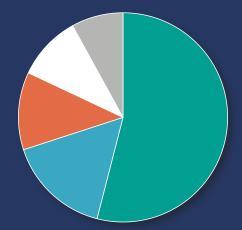
The MDS Foundation, Inc.'s Young Investigator Grant provides an investigator, aged 40 years or less, the opportunity to initiate, continue or complete a project that focuses on either basic or clinical management into the causation, epidemiology, molecular biology, cytogenetics, morphology, prognosis and treatment of the Myelodysplastic Syndromes.

# **2020 FINANCIAL** HIGHLIGHTS

The figures on this page show the financial activities of the MDS Foundation for the fiscal year ending on December 31, 2020.

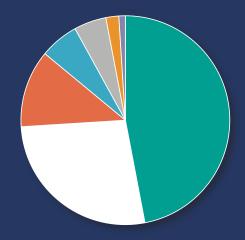
#### 2020 EXPENSES - \$2,069,831

Patient Advocacy54	<b>1</b> %
Professional Education & Training10	<b>5</b> %
Fundraising12	2%
Management10	<b>)</b> %
Research	3%



#### 2020 REVENUE - \$3,347,583

Pharmaceutical Grants	<b>47</b> %
Contributions	<b>27</b> %
Special Events	12%
Symposia Income	6%
Program Services	5%
Member Dues	<b>2</b> %
Other	1%



# **OUR SUPPORTERS**

Thank you to our 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!

AbbVie Geron Onconova Acceleron **Pfizer** Janssen Samyang **Agios** Jazz Syros **Notable Labs Astex BMS Novartis US** Taiho **DSI (Daiichi Sankyo) Novartis Global** Takeda Gamida Cell

PROUD MEMBERS:





2020 ANNUAL REPORT | 16

# **OUR TEAM**

#### **Board of Directors**

Pierre Fenaux, MD, PhD

Hôpital St Louis, Université Paris 7

Peter L. Greenberg, MD

Stanford University School of Medicine

Sandra E. Kurtin, PhD, ANP-C, AOCN

The University of Arizona Cancer Center

Alan F. List, MD

Tampa, Florida, USA

Luca Malcovati, MD

University of Pavia School of Medicine

Stephen D. Nimer, MD, Chair

Sylvester Comprehensive Cancer Center

Michael R. Savona, MD

Vanderbilt University School of Medicine

#### **EMERITUS MEMBERS**

John M. Bennett, MD

Rochester, New York, USA

Eva Hellström Lindberg, MD, PhD

Stockholm, Sweden

Charlotte M. Niemeyer, MD

Freiburg, Germany

Franz Schmalzl, MD

Innsbruck, Austria

#### **OFFICERS**

Tracey Iraca, Executive Director

Janice Butchko, Secretary

Sandra E. Kurtin, PhD, ANP-C, AOCN, Treasurer

#### **Development Board**

Stuart Goldberg, MD

John Theurer Cancer Center at Hackensack University Medical Center MDS Specialist

Jennifer Keam, MD, MPH

MDS Caregiver

Deborah Peirce, Chair

Talent/Leadership and Organizational Development Executive MDS Caregiver

Julia McGuire, Advisor

Executive Vice President , Campbell & Company Madeleine's Mom, MDS Patient

### **Medical and Scientific Advisory Board**

Rafael Bejar, MD, PhD

Moores Cancer Center at the University of California, San

Rena Buckstein, MD, FRCPC

Sunnybrook Health Sciences Centre

Mario Cazzola, MD, Chair

University of Pavia School of Medicine Fondazione IRCCS Policlinico San Matteo

Jane Churpek, MD

University of Wisconsin Carbone Cancer Center

Theo J.M. de Witte, MD, PhD

Radboud University Nijmegen Medical Centre Nijmegen Centre of Molecular Life Sciences

Erin P. Demakos, RN, CCRN

Icahn School of Medicine at Mount Sinai

Benjamin Ebert, MD, PhD

Dana-Farber Cancer Institute

Silvia M. M. Magalhães, MD, PhD

Federal University of Ceará Hospital Universitário Walter Cantídio

Moshe Mittelman, MD

Tel-Aviv Sourasky Medical Center

Yasushi Miyazaki, MD

Nagasaki University

Ghulam J. Mufti, DM, FRCP, FRCPath

King's College London & King's College Hospital

Seishi Ogawa, MD, PhD

Kyoto University

Uwe Platzbecker, MD

University Hospital Leipzig

Guillermo Sanz, MD

Hospital Universitario y Politecnico La Fe

Akiko Shimamura, MD, PhD

Boston Children's Hospital Dana-Farber Cancer Institute

Lewis R. Silverman, MD

Icahn School of Medicine at Mount Sinai

### **Foundation Staff**

Tracey Iraca, Executive Director

Janice Butchko, Project Manager

**Lea Harrison,** Senior Project Manager

Audrey Hassan, Patient Liaison

**Deborah Murray,** Administrative Support

Madelyn Geltch, Community Outreach Coordinator

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.





#### WWW.MDS-FOUNDATION.ORG

The MDS Foundation, Inc. 4573 South Broad St., Suite 150 Yardville, NJ 08620