

## MDS NEWS HIGHLIGHTS

### FROM THE GUEST EDITOR'S DESK

#### EVALUATION OF ADULTS FOR FAMILIAL MYELOID MALIGNANCY PREDISPOSITION: EYES ON THE PRIZE OF PREVENTION

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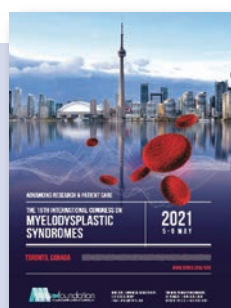


### PLAN TO ATTEND



#### ASH 2020: MDS FOUNDATION BREAKFAST SYMPOSIUM

December 4, 2020  
San Diego, California



#### 16TH INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES

May 5-8, 2021  
Toronto, Canada

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## EVALUATION OF ADULTS FOR FAMILIAL MYELOID MALIGNANCY PREDISPOSITION: EYES ON THE PRIZE OF PREVENTION



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### INTRODUCTION

Advancements in the field of familial myeloid malignancy predisposition in only a few years have been remarkable, taking us from rarely thinking about a hereditary contribution to our adult patients' histories to now a growing number of mainstream clinical guidelines including and encouraging thought of these disorders in every case.<sup>1-3</sup> Each year, the list of genes and disorders helping to explain the hereditary contribution to another subset of patients with myeloid malignancies grows,<sup>4,5</sup> and studies of the biological pathways disrupted are shedding light into early events in myelodysplastic syndromes (MDS) initiation. These are all FANTASTIC developments. However, we are still a long way off from maximizing the potential of familial myeloid malignancy predisposition assessment for MDS patients worldwide and using knowledge gained toward the ultimate goal of preventing MDS/acute myeloid leukemia (AML). Let us reflect on progress and critical needs for future progress by working through an illustrative case.

### CASE EXAMPLE

A 63-year-old man presents for hematology evaluation due to a three-month history of neutropenia (absolute neutrophil count 900,000/microliter) and

thrombocytopenia (90,000/microliter) found incidentally on laboratories done at a preventative care visit. He feels in his usual state of health and denies recent infections, bleeding, or fatigue. A bone marrow biopsy and aspirate reveal a hypocellular marrow with prominent erythroid dysplasia and 4% blasts. Karyotype is 46,XY[20].

### PROGRESS IN THE PROCESS

At face value, this case is straightforward. He now has a new diagnosis of MDS with single lineage dysplasia and can be managed accordingly. However, if his hematologist has incorporated universal family history screening into his/her clinical practice as a growing number of hematologists are, 1 out of every 7 to 9 similar adult myeloid malignancy patients will report at least one close relative with a hematologic malignancy. (unpublished data,<sup>6,7</sup>) In this particular patient's case, he was especially worried about his blood count values because he had two siblings who died of myeloid malignancies: one of MDS and the other of AML (**Figure 1A**).

Does this new information impact his diagnosis? YES. Based on the family history alone, unless there is a major known shared exposure risk factor, this family warrants a clinical diagnosis of a familial MDS/AML syndrome. Communicating this information to the pathologist who made the MDS diagnosis will change the bone marrow diagnosis line to add the following descriptor from Table 17 in the World Health Organization myeloid neoplasm classification guidelines: myeloid neoplasm with germline \_\_\_\_ mutation.<sup>1</sup> The next challenge is to find the gene that fills in that blank.

For low-grade MDS diagnoses, the pathologist may also need to reevaluate the bone marrow with this new information to ask whether an overt MDS diagnosis is warranted. Single or even multilineage

dysplasia can be observed at baseline in individuals with known familial myeloid malignancy syndromes due to germline gene defect-related abnormalities in hematopoiesis. This makes an MDS diagnosis based on dysplasia alone especially challenging and has created calls for hereditary syndrome-specific criteria for MDS diagnosis.<sup>8,9</sup>

### Patterns and Probability

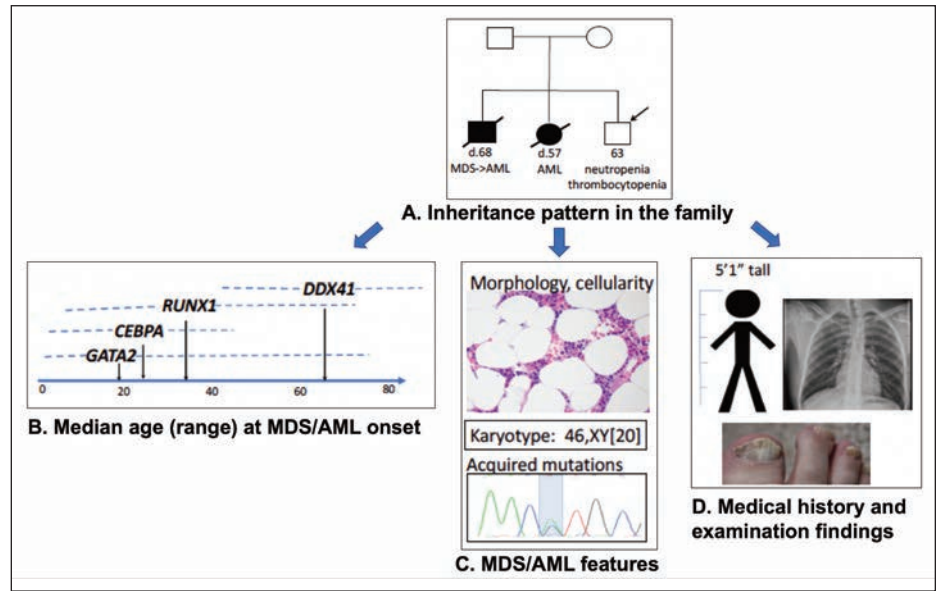
Studies of germline mutation prevalence in unselected "sporadic" MDS/AML cases are beginning to define how common hereditary myeloid malignancy predisposition syndromes are in adults. For example, a recent French series found causative germline mutations in *DDX41* in 2.4% of 1385 adults with MDS/AML.<sup>10</sup> Early data from the BEAT AML Master trial, which is attempting to collect a skin biopsy on all newly diagnosed AML patients, also found *DDX41* mutations in 2.8% of 176 patients age 60 or older.<sup>11</sup> The National MDS Natural History Study (NCT 02775383) is collecting germline samples on its planned 3500 newly diagnosed patients, which will hopefully better define prevalence estimates specifically in MDS cases.<sup>12,13</sup> Optimal germline samples, comprehensive gene lists, expert rare variant interpretation, as well as follow-up functional testing and family-based segregation studies of rare variants will be critical to maximizing information gained from these large scale studies.

Case series of families with each myeloid malignancy predisposition syndrome from different settings around the world are also helping to refine patterns suggestive of one syndrome versus others. Cytopenias at initial MDS/AML diagnosis with a bone marrow featuring hypocellularity, prominent erythroid dysplasia, and a normal karyotype are all classical findings in familial MDS/AML due to *DDX41* mutation.<sup>10,14,15</sup> For *DDX41*,

series from the United States, Japan, Taiwan, and France have demonstrated different recurrent mutation sites within the *DDX41* gene by country that could contribute to gene by ethnic background specific features.<sup>10,15-17</sup> In contrast, de novo AML with M1 or M2 morphology and double *CEBPA* mutations without preceding blood count abnormalities would have suggested familial AML due to *CEBPA* mutation.<sup>18</sup> MDS featuring dysplastic megakaryocytes, increased reticulin fibrosis, and often monosomy 7 is common in *GATA2* deficiency syndrome.<sup>19,20</sup>

While initially intimidating, recognizing these patterns can be just like a good resident morning report. “Did Mr. X have a history of lymphedema? NO. How about pulmonary fibrosis? NO....” In this process, yeses are more informative than noes because all of these disorders are incompletely penetrant for each feature. This means that a patient can still have familial platelet disorder with myeloid malignancy predisposition (FPD) even if they do not have the characteristic preceding history of thrombocytopenia or bleeding tendency. Similarly, they can have *GATA2* deficiency syndrome without a clinical history of lymphedema or warts. Arming oneself with tables from the literature,<sup>4</sup> rote memorization, or one’s own mnemonics (*GATA2* deficiency syndrome has CLAW: CMML/MDS, Lymphedema, Atypical infection, Warts) can aid in pattern recognition. Recognizing syndrome-specific features can even yield a legitimate clinical diagnosis. In fact, expert clinicians are right 91% of the time in clinically diagnosing a specific inherited bone marrow failure syndrome later confirmed molecularly through genetic testing.<sup>21</sup> With practice, you can do it too!

Going through this process for the patient above (**Figure 1A-D**), first, consider possible inheritance patterns. Keep in mind the size of the family and age of both affected and unaffected individuals, paying attention to whether the unaffected individuals have lived long enough to develop features. For our patient, his



**Figure 1.** Clinical presentation details that aid in familial myeloid malignancy syndrome diagnosis

children are too young to know what may happen to them over time. Three affected siblings could result from an autosomal dominant pattern with incomplete penetrance, meaning the parent who also carries the mutation did not develop a myeloid malignancy in their lifetime. This would be the most common pattern for adult-onset familial myeloid malignancy syndromes, but autosomal recessive or other patterns are also possible.

Next, note the median age at MDS/AML presentation (**Figure 1B**). Our patient’s family’s median is 63 years, fitting best with the median age of onset of 63 years observed in familial MDS/AML due to *DDX41* mutation versus the 19, 25, and 33 years observed in *GATA2* deficiency, familial AML due to *CEBPA* mutation, and FPD, respectively.<sup>14,15,18,22,23</sup> Presenting and historical blood counts, bone marrow cellularity, lineage-specific dysplasia(s), karyotype, and acquired gene mutations can also be helpful as noted in the patterns described above. In his case, the prominent erythroid dysplasia, hypocellular marrow with presenting cytopenias, and normal karyotype are all classical findings in familial MDS/AML due to *DDX41* mutation.<sup>14</sup> A review of systems asking key targeted questions such as the presence of anomalies, pulmonary fibrosis, and an

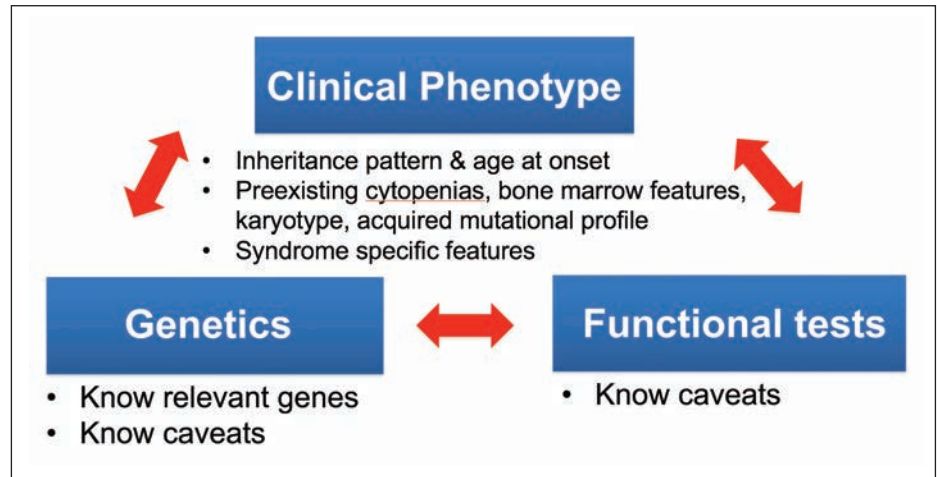
infectious disease history rounds out this evaluation.<sup>24</sup> The clinical history often has clues that are easy to overlook if not intentionally seeking them. Our patient has no notable findings, a pattern also consistent with familial MDS/AML due to *DDX41* mutation.

### Functional Testing and Genetics

For those lucky enough to have ready access to germline and acquired multi-gene tests, one may consider just skipping the memorizing and quizzing to wait for the genetic tests to provide the answer. Well, unfortunately, it’s not so straightforward. Outside of a small number of recurrent mutations affecting multiple families, the germline mutations identified in the majority of families with most of these syndromes will be unique or “private” to a single family. Thus, if clinical laboratories have never seen this variant before, all of the work that was put in characterizing the clinical phenotype above can really pay off—making a variant that would otherwise end up in the purgatory of genetics, a so-called variant of uncertain significance (“VUS”), instead be classified as a clinically useful likely pathogenic or pathogenic variant that can be acted upon. Functional tests, where available, such as chromosome breakage

testing for Fanconi anemia, can also cinch a clinical diagnosis or aid in interpretation of genetic variants identified. Thus, the sum of careful clinical phenotyping, genetic testing, and functional tests is greater than any individual part in making a diagnosis (**Figure 2**). Ongoing efforts to improve rare variant classification in familial myeloid malignancy predisposition genes take advantage of this fact, incorporating syndrome-specific clinical features and functional tests to better differentiate the benign from clinically actionable variants and hopefully minimize VUSs.<sup>25</sup>

If an acquired mutation panel is performed along with germline testing, the combined mutational pattern can further suggest a likely diagnosis. For example, if our patient had had a myeloid malignancy focused panel that included *DDX41*, we would have identified both his germline *DDX41* mutation (p.D140fs) as well as an acquired *DDX41* mutation (p.R525H), a recurrent mutation that is commonly acquired on the previously normal allele at the time of myeloid malignancy in this syndrome.<sup>26</sup> Because several of the hereditary myeloid malignancy predisposition genes are frequently mutated somatically in MDS, such as *RUNX1* and *TP53*,<sup>27</sup> these genes are already on most acquired mutation panels used for MDS diagnostic and prognostic purposes. Some centers are further adapting acquired mutation panels to specifically include additional hereditary myeloid malignancy predisposition genes that are not yet prognostic or predictive for MDS therapeutic purposes but instead to screen for a hereditary etiology. Although most of the mutations found in an MDS sample will be acquired, *DDX41* is different because the majority of the mutations found in this gene are germline.<sup>10,28</sup> Thus, consenting patients to MDS “acquired” panels, especially if adapted for screening the germline, should acknowledge this intention. Overall, paired germline and acquired multi-gene testing along with clinical phenotyping and functional testing have the greatest chance of providing a molecular diagnosis to inform management



**Figure 2.** Familial myeloid malignancy predisposition work-up elements: the sum is greater than its parts

for the patient and identify those at risk in the family for counseling and risk management.

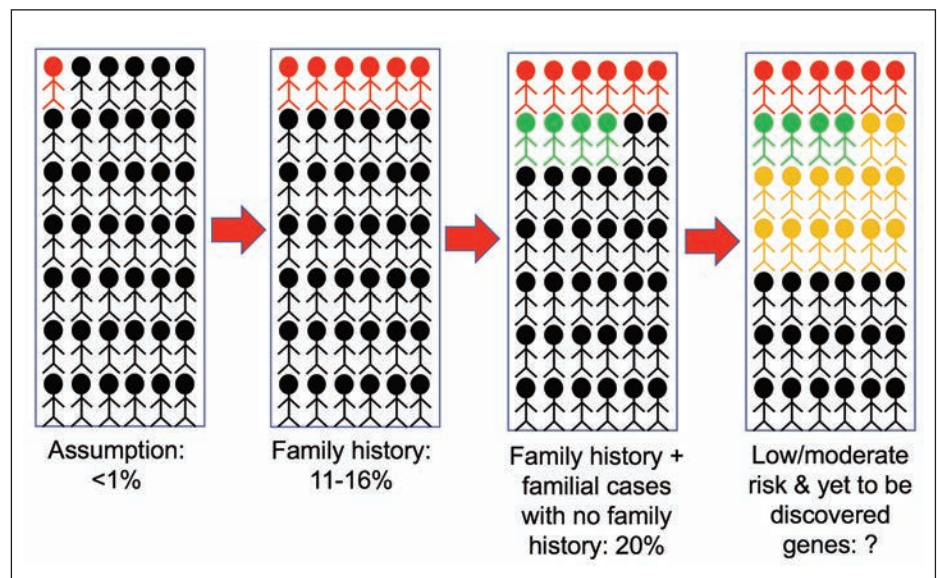
### CRITICAL NEEDS TO KEEP MOMENTUM GOING

Adult hematology is experiencing a major paradigm shift going from the assumption that familial predisposition is exceedingly rare (**Figure 3**) to growing recognition by both patients as well as clinicians at the time of diagnosis that the family history is important. A second paradigm shift is also occurring. This one involves recognition that paired germline and acquired genetic assessment at

diagnosis can impact the interpretation of blood and bone marrow findings, the official World Health Organization classification, and management of MDS patients. Critical needs to keep the momentum going and maximize potential benefit of familial myeloid malignancy predisposition assessment for clinical care and science are outlined below.

### Education

First and foremost, investment in hereditary hematology-focused educational opportunities for diverse disciplines, such as genetic counselors, medical geneticists, benign and malignant hematologists,



**Figure 3.** Estimated proportion of MDS cases with an underlying inherited risk component

primary care providers, nurses, and advanced practitioners, is crucial to helping more patients with MDS or who are at risk for MDS be recognized. With more practitioners comfortable with the spectrum of hematologic diagnoses and vocabulary and aware that it is possible to have hereditary forms of blood disorders presenting across the lifespan, it is more likely that each patient bringing their own personal or family history of blood disorders to attention will be acknowledged and given the opportunity to pursue the etiology. A formal genetic hematology fellowship for hematologists, geneticists, and/or bioinformaticians in training is possible and would foster talent development for future discoveries and improvements in clinical care and implementation.

### Access

Access to both germline and acquired genetic testing in MDS must be improved. Access in this context is multipronged from access to clinicians at the bedside who recognize whom to test and either are comfortable with informed consent and navigating the entire testing process or are willing to make the connection to someone who is. Access also requires the ability to collect appropriate samples, such as skin fibroblasts or hair follicles for germline tests, as well as laboratories providing affordable assays with comprehensive and up to date gene lists, focused hereditary and acquired hematology genetics expertise to maximize interpretation potential, and turnaround times relevant for the care of patients with urgent diagnoses. Access also requires financial investment from insurance companies, hospitals, and/or governmental agencies willing to provide coverage to pay for testing to avoid financial toxicity to patients. Finally, access requires systems change to allow all of this to happen seamlessly in the clinic so it can more easily be incorporated into already complex care needs and make it more likely that many practitioners can adopt genetic testing into their own practices.

### Collaboration

Inherent in understanding the clinical and functional significance of each individual genetic variant is studying it in as many people in diverse ethnic backgrounds as possible. However, with increasing regulation and restrictions being placed on data and sample sharing across international lines, our field will need to get creative to maximize utility of data gathered from individual families across the world. Formulating and adhering to consensus clinical care guidelines that can then be regularly reassessed with objective data would hasten evidence-based clinical management progress. Clinical and research teams agreeing on what key variables to collect over time and uniform data formats would make analysis of data across sites easier. Adopting methods used in other fields, like federated data networks that allow analysis of data across sites without actually moving the data out of an individual site, may be helpful. Seeking out and encouraging thought leaders in fields like data science and engineering to get involved in hematology could further inform new methods.

### Research

Many key questions still remain to be answered. Clinically, despite a growing gene list, only about 30% of familial MDS/AML cases like our case presented here will be explained by currently known syndromes.<sup>29</sup> Thus, what other genes and their variants contribute to MDS/AML risk (**Figure 3**)? In familial MDS/AML as in other hereditary cancer syndromes such as hereditary breast cancer susceptibility, the genes with the largest effect on susceptibility and those that account for the greatest proportion of cases will be the easiest to find. Figuring out the very rare or the variants that only increase risk by a small to moderate amount and are, therefore, more common in the population will be much harder. For example, heterozygous pathogenic or likely pathogenic variants in genes that usually cause MDS/AML predisposition in the homozygous or compound heterozygous

state, such as those in the Fanconi anemia or telomere biology disorder pathways or even SBDS are being found more frequently than expected by chance in patients with MDS/AML.<sup>30</sup> Are these actually contributors to MDS/AML risk and if so, by how much do they increase risk?

Biologically, why does a particular germline mutation increase risk of MDS/AML and by how much is the lifetime risk elevated? What is/are the triggering event(s) that lead from the at-risk state to overt MDS/AML? From what source(s) do these triggers arise (e.g. are DNA damaging events due to endogenous cell metabolism-related exposures or are they from external exposures like smoking)? How can we intervene on this process to prevent MDS/AML or at least slow progression?

A few recent studies have begun to shed light on effects of the mutations themselves. For example, clonal hematopoiesis (CHIP) is a known marker of future MDS/AML risk.<sup>31</sup> However, the actual initiating events in this process have remained unclear. A recently described autosomal recessive familial MDS/AML syndrome caused by biallelic or compound heterozygous mutations in *MBD4* has highlighted the role of DNA repair on CHIP initiation and MDS/AML risk.<sup>32</sup> Specifically, *MBD4* is a DNA glycosylase involved in repairing methylation damage through the base excision repair pathway. Without this gene, there is a remarkable accumulation of C>T transition mutations. Interestingly, *DNMT3A* appears particularly affected with multiple different *DNMT3A* clones found in the blood and a resulting AML with myelodysplastic related changes with a remarkably high mutational burden overall. This syndrome is unique in its more direct link to a specific DNA repair deficit as a major contributor to the CHIP initiating event(s). Recent work in several other inherited bone marrow failure syndromes has also highlighted syndrome-specific CHIP features that inform underlying pathogenesis. For example, a high proportion of individuals with Shwachman Diamond syndrome develop TP53-mutated

CHIP, often with multiple different *TP53* mutated clones, by the age of 20<sup>33</sup> whereas individuals with FPD who carry a germline *RUNX1* mutation also develop early-onset CHIP, but involving many different genes.<sup>29</sup> Further study into the exact inciting events in CHIP initiation whether stochastic or exacerbated by specific endogenous or exogenous exposures will undoubtedly advance our understanding of pathogenesis and get us closer to the goal of prevention.

## CONCLUSIONS

Translation to improving familial myeloid malignancy predisposition assessments and syndrome-specific care for all patients in diverse, real-world, international clinical practice settings will take major efforts to improve access, education, clinical tools, policy, and collaboration. Forward progress on many of these areas is noticeable and provides hope that we will reach a future where prevention of MDS/AML is possible.

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## MEETING HIGHLIGHTS AND ANNOUNCEMENTS

62ND ASH ANNUAL MEETING & EXPOSITION • DECEMBER 4, 2020 • SAN DIEGO CONVENTION CENTER, SAN DIEGO, CALIFORNIA

BREAKFAST SYMPOSIUM • DECEMBER 4, 2020

### ADVANCES IN OUR UNDERSTANDING OF THE PATHOPHYSIOLOGY AND MANAGEMENT OF MYELOYDYSPLASTIC SYNDROMES

SAVE THE DATE

SAN DIEGO, CALIFORNIA



CONTINUING OUR MISSION OF INTERNATIONAL COLLABORATION IN THE FIELD OF MDS

On behalf of the MDS Foundation and our Board of Directors,  
**THANK YOU** for joining our Breakfast Satellite Symposium!



*Our professional MDS Symposium at the American Society of Hematology Conference – A great start!!*

#### BREAKFAST SYMPOSIUM

### PRECISION HEMATOLOGY IN MYELOYDYSPLASTIC SYNDROMES

DECEMBER 6, 2019

7:00 – 11:00 am

Orange County Convention Center  
Orlando, Florida



THE PRESENTATIONS ARE NOW AVAILABLE ON OUR WEBSITE AT  
<https://www.mds-foundation.org/ash-2019-presentations/>

## 2ND REGIONAL SYMPOSIUM ON MDS – ISRAEL 2020



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<https://www.mds-foundation.org/professional-learning-center/#2ndregionalsymposium>

### UPCOMING MEETING

## XVI URUGUAYAN HEMATOLOGY CONGRESS



**RADISSON HOTEL**  
**MONTEVIDEO – URUGUAY**



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**POSTPONED**  
**TO SEPTEMBER 1, 2021**

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*XI Uruguayan Hematology Congress*

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## LATEST NEWS REGARDING THE MOLECULAR MUTATION PROJECT OF THE IWG-PM

### IWG-PM/MOLECULAR PROJECT

In patients with MDS, *TP53* mutations associate with high-risk presentation, complex karyotype, acute myeloid leukemia (AML) progression and poor response to hematopoietic stem cell transplantation. These findings highlight the relevance of *TP53* as a prognostic and predictive biomarker. Despite the central role of *TP53* in MDS, the clinical implications of *TP53* mutations in the context of allelic state have not been extensively studied. Under the auspices of the International Working Group for Prognosis in MDS Molecular project (IWG-PM/Molecular) investigational efforts generated data from which an abstract describing results obtained from sequencing marrow or blood samples from a representative cohort of 3,324 pre-diagnosis MDS patients on a next generation sequencing (NGS) panel along with a validation cohort from a Japanese MDS sample compendium.<sup>1</sup>

Data analysis of this study segregated patients into two *TP53* states: a mono-allelic state where one wild type allele remained (33% of *TP53* mutated patients, n=126); and a multi-hit state where *TP53* was altered multiple times by either mutations, deletions or cnLOH (67% of *TP53* mutated patients, n=254). The findings demonstrated that *TP53* state was associated with clinical presentation and outcomes. Mono-allelic *TP53* mutation patients presented with more favorable disease than multi-hit *TP53* mutated patients. Using multivariate models that considered age, peripheral blood counts, blasts and IPSS-R cytogenetic score, multi-hit *TP53* mutation state rather than the mutation alone was



an independent prognostic factor for overall survival and AML transformation, whilst mono-allelic *TP53* state was not. Mono-allelic *TP53* patients had more favorable disease than multi-hit *TP53* patients and were enriched in low risk WHO subtypes. Critically, multi-hit *TP53* associated with worse overall survival as compared to mono-allelic *TP53* and with more pronounced AML transformation. These findings indicated that *TP53* status is a critical candidate for incorporation into molecularly informed risk stratification schemas (molecular IPSS-R). Thus, *TP53* mutation state is important for MDS risk estimation, disease monitoring and future correlative research.

The IWG-PM/Molecular group project is ongoing with plans for further development of a global classification schema for MDS (IPSS-R/molecular). This issue was further discussed at the MDS Foundation Symposium at ASH 2019.<sup>2</sup>

### SF3B1 MUTATED MDS AS A DISTINCT DISEASE SUBTYPE PROJECT

A large body of evidence indicates that in MDS, the *SF3B1* mutation is closely associated with ring sideroblasts and ineffective erythropoiesis and is characterized by less aggressive clinical course. The available evidence supports



recognition of *SF3B1*-mutant MDS as a distinct nosologic entity. To further validate this, Dr. Malcovati and colleagues interrogated the dataset of the IWG-PM. Based on these findings, the following diagnostic criteria for the MDS with mutated *SF3B1* was proposed as a distinct MDS subtype: (i) cytopenia defined by standard hematologic values; (ii) somatic *SF3B1* mutation; (iii) isolated erythroid or multilineage dysplasia; (iv) the presence of any ring sideroblasts; (v) bone marrow blasts <5% and peripheral blood blasts <1%.<sup>3</sup>

### IWG-PM: THERAPY-RELATED MDS PROJECT

In the current WHO classification, therapy-related Myelodysplastic Syndromes (t-MDS) are placed together with therapy-related Acute Myeloid Leukemia (t-AML) and Myeloproliferative Neoplasms (t-MPN) into one subgroup (t-MN) independent of morphological or prognostic features. Drs Kuendgen, Nomdedue, Tuechler and colleagues have assembled a database including 2,087 t-MDS patients from different international MDS groups to evaluate current classification and prognostic tools.<sup>4,5</sup> Cytogenetic data was reviewed and strict selection criteria with regard to completeness were applied. They analyzed and compared 1,245 t-MDS patients to 4,593 primary MDS (p-MDS) patients represented in the IWG-PM database. These data demonstrated that the IPSS-R and WPSS-R separated t-MDS patients into differing risk groups effectively and indicated that all established prognostic risk factors for p-MDS maintained relevance for t-MDS, with cytogenetic features having enhanced predictive power. Poorer clinical outcomes occurred in each t-MDS

compared to p-MDS subgroups. Despite t-MDS being considered as having a uniformly poor prognosis these data demonstrated differing outcomes for each t-MDS subgroup. Given these data, these findings suggest the ability and need for classifying t-MDS as a separate entity and distinct from the other WHO classified t-myeloid neoplasms (t-MNs). This terminology would enable more reasonable treatment decisions and facilitate the inclusion of t-MDS patients into clinical studies.

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4. Kuendgen A, Tuechler H, Nomdedeu M, et al. Therapy-related MDS can be separated into different risk-groups according to tools for classification and prognostication of primary MDS. *Proc ASH*, San Diego, December 2018, #3103.
5. Nomdedeu M, Tuechler H, Kuendgen A, et al. Cytogenetic findings in therapy-related MDS – relation with primary disease and therapy. *Proc MDS Foundation Symposium*, Copenhagen, May 2019.



This global project is being coordinated by Ben Ebert and Peter Greenberg (co-Chairs), Rafael Bejar and Ellie Papaemmanuil, with statistical support by Donna Neuberg, Kristin Stevenson and Heinz Tuechler.



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# INTERNATIONAL WORKING GROUPS

MAKING CONNECTIONS AROUND THE WORLD

## THE MDS/MPN INTERNATIONAL WORKING GROUP



**MICHAEL SAVONA**

*Vanderbilt University Medical Center  
Nashville, Tennessee*

The first investigator driven, multi-national MDS/MPN-specific trial in the world is led by the MDS/MPN IWG. **ABNL MARRO – A Basket Trial of Novel therapy combinations in untreated MDS/MPN And Relapsed/Refractory Overlap Syndromes (ABNL-MARRO)** – is an international study designed to quickly test new compounds and combinations of therapy at referral centers within the MDS/MPN IWG 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 has been approved by the FDA. ABNL MARRO-001 uses an oral DNA methyltransferase inhibitor (DNMTi), ASTX727, as a backbone with combination therapies targeting JAK1, PIM kinase and LSD-1. DNMTi combinations have been evaluated in phase 1 safety studies, and will form 3 separate arms in ABNL MARRO-001. With ABNL MARRO-001, the MDS/MPN IWG aims to validate the proposed criteria for response in MDS/MPN, test QOL tools in patients with MDS/MPN,

develop new biomarkers for response to therapy, and augment efforts of large scale prospective genotyping efforts in MDS/MPN. This infrastructure will allow for ABNL-MARRO-002, -003, and so on, to quickly offer new therapies to patients with MDS/MPN.



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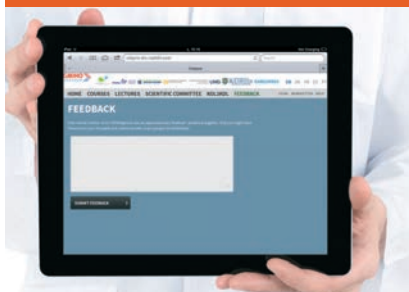
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## DO YOU KNOW YOUR MDS SUBTYPE AND IPSS-R SCORE?

We launched a recent survey which showed that only 50% of patients know their subtype and only about 30% know their IPSS-R Score. Your subtype and IPSS-R score determine your personalized treatment plan.

*Knowing your subtype and IPSS-R score can help guide discussions with your doctor.*

## KNOWLEDGE IS POWER



[www.mds-foundation.org](http://www.mds-foundation.org)

## ASK YOUR HEALTHCARE TEAM...



Knowing your IPSS-R score and MDS subtype can guide discussions with your healthcare team about the best treatment options for you!

## KNOW YOUR SCORE

The IPSS-R is a classification system used by doctors to help predict a person's risk of developing AML and overall survival without treatment.

### CATEGORIES & SCORES

Very Low .....	≤1.5
Low .....	>1.5 - 3
Intermediate .....	>3 - 4.5
High .....	>4.5 - 6
Very High .....	>6

## KNOW YOUR SUBTYPE

MDS is classified into several different subtypes based on the following features:

- Blood cell counts
- Percentage of blasts in the bone marrow
- Cytogenetics

### FAB SUBTYPES

Refractory Anemia (RA)  
Refractory Anemia with Ringed Sideroblasts (RARS)  
Refractory Anemia with Excess Blasts (RAEB)  
Refractory Anemia with Excess Blasts in Transformation (RAEB-t)  
Chronic Myelomonocytic Leukemia (CMML)  
Acute Myeloid Leukemia (AML)

### WHO SUBTYPES

MDS with single lineage dysplasia  
MDS with ring sideroblasts (MDS-RS)  
MDS-RS and single lineage dysplasia  
MDS-RS and multilineage dysplasia  
MDS with multilineage dysplasia  
MDS with excess blasts  
MDS with isolated del(5q)  
MDS, unclassifiable  
Provisional entity:  
Refractory cytopenia of childhood

Calculate your IPSS-R Score and IPSS-R Category

Variables (units) [usage range]	Value
<b>Hemoglobin</b> 9g/dl0. [4-20] A possible conversion for Hb values: 10 g/dL=6.2 mmol/L, 8g/dL=5.0 mmol/L	<input type="text"/>
<b>Absolute Neutrophil Count</b> (x10 <sup>9</sup> /L). [0-15]	<input type="text"/>
<b>Platelets</b> (x10 <sup>9</sup> /L). [0-2000]	<input type="text"/>
<b>Bone Marrow Blasts</b> (percent). [0-30]	<input type="text"/>
<b>Cytogenetic Category</b> <input type="radio"/> Very Good <input type="radio"/> Good <input type="radio"/> Intermediate <input type="radio"/> Poor <input type="radio"/> Very Poor	
<input type="button" value="calculate &gt;"/>	

More detailed information on IPSS-R scores and subtype can be found online in our Building Blocks of Hope resource.

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### POSTPONED – SPRING MDS AWARENESS WALKS

Dear Friends of the MDS Foundation,

We understand that during the uncertainty of this pandemic, the main concern of all is protecting yourself as well as your families and friends from exposure to the COVID-19 virus. The highest priority of the MDS Foundation at this time, and always, is the health and safety of our community. With this in mind, and in consultation with medical professionals, we have decided to postpone our upcoming MDS Awareness Walks in Chicago and New York City. We are waiting for permits to be finalized, but we are hoping to reschedule the walks for early Fall. Look to our website and facebook for updates on our MDS Awareness Walks which may be going virtual due to the coronavirus pandemic. We can't come together as a community in person, but that doesn't mean we can't make a difference individually. This situation is continuously evolving and we will evaluate any further developments as they come. We will send out another email once we have confirmed the new dates. In the meantime, should you want to support the walks or set up a team, please visit <https://www.mds-foundation.org/mds-awareness-walk/>.

We appreciate your continued support and thank you for understanding as we navigate the current situation.

Best wishes during this difficult time.

Tracey Iraca, Executive Director

### HIGHLIGHTS OF LATEST LITERATURE IN MDS

SUNEEL D. MUNDLE, PHD  
RHEA MUNDLE

Listed below are citations of some new publications relevant to MDS (pathogenesis, clinical characterization, management, etc.). To access the complete articles log on to [www.pubmed.gov](http://www.pubmed.gov).

#### EPIDEMIOLOGY, DIAGNOSIS AND PROGNOSIS:

1. Liang S, et al. Prognostic value of DNMT3A mutations in myelodysplastic syndromes: a meta-analysis. *Hematology*, 2019;24(1):613–622. (<https://pubmed.ncbi.nlm.nih.gov/31482762/>)  
*The meta-analysis included 12 studies with 2236 MDS patients. A pooled HR for overall survival and leukemia free survival showed significantly adverse prognostic impact of DNMT3A mutations, (OS- HR-1.65,  $p<0.001$ ; LFS- HR-4.62,  $p<0.001$ )*
2. Kurumaddali A, Salem AH and Agarwal AK. Spanish guidelines for the use of targeted deep sequencing in myelodysplastic syndromes and chronic myelomonocytic leukemia. *J Cancer*, 2019;10(22):5427–5433. (<https://pubmed.ncbi.nlm.nih.gov/31632487/>)  
*Using a pre-established clinical trial database and linear regression analysis of 25 trials with 38 cohorts, the authors found that  $\geq$ PR (PR+CR+Marrow CR) was a strong predictor of OS (adjusted  $R^2=0.64$ ). Furthermore, for a given response rate ( $\geq$ PR) or treatment group, the survival was longer in treatment naïve vs previously treated patients.*
3. McDonald LS, et al. A multicenter report on the natural history of myelodysplastic syndromes in very old patients (aged over 85 years). *Leuk Lymphoma*, 2019; 60(5):1324–1327. (<https://pubmed.ncbi.nlm.nih.gov/30632846/>)



- Data on 47 men and 37 women aged  $\geq 85$  yrs demonstrated that 93% patients were anemic at diagnosis with a med Hb level of 9.5g/dL. Sixty four percent of patients had more than one cytopenia, 53% patients had elevated serum ferritin levels, and 88% patients had one or more significant comorbidities (cardiac, renal, or diabetes). Of the patients with significant anemia (Hb  $<10$  g/dL), 42% received erythropoietin. Of the 29 transfusion-dependent patients only 48% received erythropoietin. The AML transformation rate was 7%. The median overall survival was 16 mo in men and 27 mo in women. In 34 patients with IPSS-R classification, the med survival was 31.5 mo in very good risk, 41.5 mo in good risk, and 8mo in int risk patients.*
4. Starkman R, et al. An MDS-Specific frailty index based on cumulative deficits adds independent prognostic information to clinical prognostic scoring. *Leukemia*, 2019;Dec 6 [Online ahead of print]. (<https://pubmed.ncbi.nlm.nih.gov/31811236/>)  
*A frailty Index (FI) based on impact of deficits on likelihood of adverse outcomes was calculated in 440 MDS patients. The median FI was 0.25 and FI was correlated with age, IPSS-/R risk. The FI  $\leq 0.2$  vs  $>0.2-0.3$  vs  $>0.3$  demonstrated significantly different OS with a median 55.6 mo, 24 mo and 10.9 mo respectively ( $p<0.0001$ ). FI*

*remained as a significant covariate of OS in a multivariate analysis as well. Additionally, FI discriminated OS within each of the IPSS-/R categories.*

#### TREATMENT:

##### RBC Transfusion and Growth Factors:

1. Stanworth SJ, et al. Red cell transfusion in outpatients with myelodysplastic syndromes: A feasibility and exploratory randomized trial. *Br J Haematol*, 2020; Jan 20 [Online ahead of print]. (<https://pubmed.ncbi.nlm.nih.gov/31960409/>)  
*This multinational study randomized 38 patients in outpatient clinic to initiate RBC transfusion at standard threshold of Hb  $\leq 8.0$  g/dL ( $n=20$ ) vs a liberal threshold of Hb  $\leq 10.5$  g/dL ( $n=18$ ). The compliance for transfusion threshold was 86% vs 99% in the standard vs liberal groups and the mean pre-transfusion Hb was 8.0 g/dL and 9.7 g/dL respectively. Using EORTC QLQ-C30 and EQ-5D-5L tools, the quality of life was found to be better in patients on liberal Hb threshold arm.*
2. de Swart L, et al. Impact of red blood cell transfusion dose density on progression free survival in lower-risk myelodysplastic syndromes patients. *Haematologica*, 2020;105(3):632–639. (<https://pubmed.ncbi.nlm.nih.gov/31171638/>)  
*The analysis based on European MDS registry included 1267 lower risk MDS*

patients: 317 had died without progression and 162 had disease progression. When assessed for the impact of blood transfusions on overall progression-free survival (PFS), the transfusion density as low as 3 units/16 weeks was associated with poorer PFS that continued to increase beyond 8 units/16 weeks.

3. Kaphan E, et al. Impact of transfusion on survival in patients with myelodysplastic syndromes: current knowledge, new insights and transfusion clinical practice. *Blood Rev*, 2019; Dec 18 [Online ahead of print]. (<https://pubmed.ncbi.nlm.nih.gov/31918886/>)

Patients treated with red blood cell (RBC) transfusions experience shorter survival as compared to those treated with erythropoiesis stimulating agents (ESAs). The report has attempted to list the physiologic impacts of chronic RBC transfusion strategy highlighting iron toxicity, cardiac event rate, oxidative stress and RBC storage lesions due to physicochemical changes in RBCs.

4. Swaminathan M, et al. A phase 2 clinical trial of eltrombopag for treatment of patients with myelodysplastic syndromes after hypomethylating agent failure. *Leuk Lymphoma*, 2019; 60(9):2207–2213. (<https://pubmed.ncbi.nlm.nih.gov/30773968/>)

Eltrombopag was given at a starting dose of 200mg orally per day either as monotherapy (n=7) or was added to HMA (n=22) after the failure of initial HMA therapy of high risk MDS. There was one early death (<30 days) due to infection/sepsis, an overall 11% (3/28) platelet improvement rate and a med survival of 12 mo. Addition of eltrombopag to HMA did not show additive toxicity.

### Hypomethylating Agents:

1. Mozesohn L, et al. Healthcare utilization in patients with higher-risk MDS/low-blast count AML treated with azacytidine in the real-world. *Leuk*

*Lymphoma*, 2020; Feb 8 [Online ahead of print]. (<https://pubmed.ncbi.nlm.nih.gov/32036719/>)

The Ontario AZA-MDS registry linked to population based administrative databases showed approx. 80% patients (705/877) had at least one emergency clinic visit; 33% in their first cycle. Also, approx. 77% had at least one hospitalization with a mean length approx. 18 days. A greater comorbidity, non-response to AZA and transfusion dependence were associated with both emergency room visits and hospitalizations. Also, among patients with  $\geq 3$  cycles of treatment hospitalization during first cycle was associated with an increased risk of death.

2. Chandhok N, et al. Hypomethylating agent-based combinations in higher risk myelodysplastic syndrome. *Leuk Lymphoma*, 2019; Dec 9 [Online ahead of print]. (<https://pubmed.ncbi.nlm.nih.gov/31814484/>)

The authors argue that there continues to exist an unmet need to improve upfront treatment of higher risk MDS patients as only a proportion of patients respond to hypomethylating agents and the responses are less durable. Authors propose upfront combinations with other agents as a way to improve outcomes of HMA and list venetoclax and rigosertinib as promising agents for such combination.

3. Ren Y, et al. Decitabine for myelodysplastic syndromes: Dose comparison in a real world clinical setting. *Leuk Lymphoma*, 2019;60(7): 1731-1739. (<https://pubmed.ncbi.nlm.nih.gov/30616472/>)

This was a retrospective study with 133 MDS patients. The study demonstrated that in real world patients 15 mg/m<sup>2</sup>/day dose vs. 20mg/m<sup>2</sup>/day dose resulted in a comparable response rates (ORR-51.8% vs 52%, CR-15.7% vs 22% respectively), longer survival (21.6 mo vs 15.2 mo respectively), and

lower hematologic toxicity (Gr3/4 neutropenia – 60% vs 88%; thrombocytopenia – 65% vs 88% respectively). Additionally, the report also found that the overall survival benefit with 15 mg/m<sup>2</sup>/day was primarily associated with lower risk patients.

4. Ades L, et al. A phase II study of efficacy and safety of an intensified schedule of azacytidine in intermediate-2 and high-risk patients with myelodysplastic syndromes: A study by the Groupe Francophone Des Myelodysplasies (GFM). *Haematologica*, 2019;104(4):e131–e133. (<https://pubmed.ncbi.nlm.nih.gov/30381302/>)

An azacytidine treatment intensification tested using a schedule leading to 20% higher number of days with azacytidine (75 mg/m<sup>2</sup>/day x 5 days in 14-day cycle) administration during first 8 weeks of treatment. Patients who achieved CR/PR received additional aza at 21-day cycles x 4 followed by std 7-day dosing in a 28-day cycle until progression. For patient not achieving CR or PR after first 8 weeks, additional 8 weeks of intensified treatment was given. The primary end point was response by IWG 2006 criteria after first 4 and 8 dose intensified cycles (15-day cycles). The int-2- and high-risk proportions were 65:35 among the 26 evaluable patients. The overall marrow response after 4- or 8-weeks intensified treatment was 22% (6/27). The marrow CR+ hematologic improvement (HI) was 65% after week 4 and 62% after week 8. With a f/u of approx. 42 mo, median duration of CR/PR was 10.5 mo, duration of overall response was 14 mo, and overall survival was 21.5 mo. Treatment intensification was not associated with increased toxicity.

5. Zeidan A, et al. Treatment sequence of lenalidomide and hypomethylating agents and the impact on clinical outcomes for patients with myelodysplastic syndromes. *Leuk Lymphoma*,

2019;60(8):2050–2055. (<https://pubmed.ncbi.nlm.nih.gov/30636526/>) A US claims database study (Inovalon MORE2 registry) comparing outcomes of treatment sequence Len- HMA vs HMA-Len demonstrated longer time to discontinue second treatment (HR-0.52,  $p=0.023$ ), and to disenroll from insurance used as proxy for survival (HR-0.64,  $p=0.017$ ).

### Novel Therapies:

1. Sekeres M and Steensma D. Rethinking clinical trial end points in myelodysplastic syndrome. *Leukemia*, 2019; 33(3):570-575. (<https://pubmed.ncbi.nlm.nih.gov/30700839/>)  
The review evaluates challenges to drug development and emphasizes the meaningfulness of study end points relevant to patients. The report also underscores the importance of assessing longer term end points such as response duration, quality of life and overall survival in addition to the currently common end points of hematologic and bone marrow response rates.

### PATIENT REPORTED OUTCOMES:

1. Barot SV, Patel BJ, and Gerds AT. Patient-reported outcomes in myelodysplastic syndromes: the move from life span to health span (MDS). *Current Hematol Malig Rep*, 2020; Feb 11 [Online ahead of print] (<https://pubmed.ncbi.nlm.nih.gov/32048198/>)  
Although PRO measurement is less common in clinical studies in MDS, when conducted have demonstrated positive results with growth factors, HMS and IMiDs. Many new clinical studies have begun to incorporate a prospective PRO assessment using broad metrics such as EORTC-QLQ-C30, the previously reported FACT-An and MDS specific tools like QUALMS (Quality of life in Myelodysplasia Scale).

### PATHOBIOLOGY:

1. Elvarsdóttir EM, et al. A three dimensional in vitro model of erythropoiesis recapitulates erythroid failure in myelodysplastic syndromes. *Leukemia*, 2020;34(1):271–282. (<https://pubmed.ncbi.nlm.nih.gov/31375745/>)  
Using a modified scaffolding of collagen coated polyurethane 3D cultures were established with mononuclear cells (MNC) and CD34+ enriched bone marrow cells from healthy volunteers and MDS-RS patients. Such 3D cultures supported proliferation and differentiation of erythroid cells from CD34+ cells and multilineage growth from MNC for more than 4 weeks. Importantly, the cultures demonstrated significant erythroid differentiation including erythroid islands and enucleated erythrocytes, preserved RS phenotype and the SF3B1 mutated clone. This technology could have significant implication in drug development for MDS.
2. Behbehani GK, et al. Profiling myelodysplastic syndromes by mass cytometry demonstrates abnormal progenitor cell phenotype and differentiation. *Clin Cytometry*, 2020; 98:131–145. (<https://pubmed.ncbi.nlm.nih.gov/31917512/>)  
A high-dimensional single cell mass cytometry (MCM) in addition to previously reported surface anomalies also detected increased CD321 and CD99 expression and decreased CD47 expression. In hematopoietic between MDS vs cytopenia of undetermined significance and finally the high-parameter MCM data identified abnormal differentiation patterns of myeloid cells that were immunophenotypically closer to myeloid derived suppressor cells.

### REVIEWS, PERSPECTIVES & GUIDELINES:

The following articles provide significant review of literature and/or innovative perspective on the state-of-the-art in MDS or discuss therapeutic management guidelines and identify need for additional prospective studies.

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A special thanks to Suneel and Rhea Mundle for their great efforts in monitoring these important MDS peer-review publications.

### ERYTHROPOIESIS AND RING SIDEROBLASTS: WHAT DOES THAT MEAN FOR MDS PATIENTS?



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Erythropoiesis is the term for producing red blood cells from the hematopoietic stem cells to circulating mature red blood cells. This occurs in the bone marrow, and is quite a complex process producing over 10 billion red blood cells hourly under normal conditions to maintain stable hemoglobin levels.<sup>1</sup> In myelodysplastic syndromes (MDS), erythropoiesis is defective resulting in anemia; a hallmark of the disease.<sup>2</sup> The majority of patients diagnosed with MDS will become dependent on red blood cell transfusions.<sup>3</sup> One of the goals of treatment with lower-risk MDS (LR-MDS), defined as very low, low, or intermediate on the revised international prognostic scoring system (<3.5 points)<sup>4</sup>, is to minimize the number of red blood cell transfusions and improve quality of life.

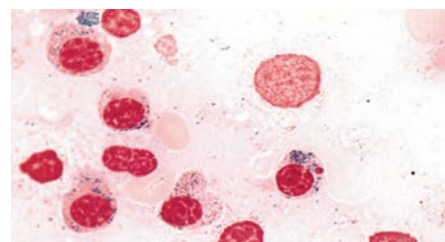
Current treatments for LR-MDS are focused on treatment of anemia with recent improvement in the understanding of ineffective erythropoiesis. There are two areas of primary interest, early stage and a later stage, focused on terminal erythroid maturation into red blood cells.<sup>5</sup> Erythroid stimulating agents (ESAs), such as epoetin alfa and darbepoetin alfa, are frequently first-line treatment for anemia in LR-MDS, targeted toward early stage erythropoiesis. Specific criteria are used to determine patients who will likely benefit from ESAs. Criteria include low transfusion burden (<2 units per month) and an erythropoietin level < 500 u/L.<sup>6</sup> Response rates to ESAs

overall are between 20 and 40%. Patients usually respond for 18–24 months. For patients with deletion of 5q, lenalidomide is the standard of care with a 67% response rate that normally lasts about 3 years.<sup>7</sup> Various sites of differentiation have been postulated for lenalidomide.<sup>8</sup>

On April 3, the Food and Drug Administration approved luspatercept (REBLOZYL, Celgene Corporation) for the treatment of anemia in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T) who have failed treatment with erythroid stimulating agents and required 2 or more units of packed red blood cells in an 8-week period. Luspatercept works further along in the pathway of red blood cell development than prior treatments. In clinical trials, side effect profile included fatigue, diarrhea, nausea, asthenia, dizziness and back pain.<sup>9</sup> Luspatercept is given subcutaneously every 3 weeks and is dosed at 1 mg/kg. Response rates varied among the clinical trials for reduction in number of red blood cells transfused. However, approximately 40% of patients experienced improvement with reduction in the number of red blood cells needed. This is an exciting time with a new treatment now available for the treatment of anemia in MDS!

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**Ring Sideroblasts in MDS**  
(courtesy of ASH image bank)

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## CORD BLOOD REGISTRY

### CORD BLOOD REGISTRY

#### CORD BLOOD REGISTRY: PROVIDING CORD BLOOD BANKING TO FAMILIES IN NEED

**ELIZA STROH, MS, LCGC**

*Cord Blood Clinical Specialist  
at Cord Blood Registry*

Cord blood is a rich source of blood forming stem cells (called hematopoietic stem cells or HSCs). For more than 20 years, cord blood has been used as a source of stem cells for patients requiring a stem cell transplant as part of their treatment for bone marrow failure conditions, like the myelodysplastic syndromes (MDS), as well as other hematological diseases and malignancies. To date, more than 40,000 cord blood stem cell transplants have been performed worldwide. In transplant medicine, HSCs are used to regenerate the patient's blood and immune systems after chemotherapy and/or radiation treatments have been given to suppress or ablate the patient's own blood and immune systems.

Cord blood HSCs have advantages over other sources of HSCs, such as those derived from bone marrow, including a lower risk of graft-vs-host disease, which can be a serious side effect of HSC transplants.

Depending on the condition being treated, the stem cells used in a transplant may come from a healthy donor or from the patient themselves. In treating patients with MDS, the stem cells typically come from a healthy donor, preferably a sibling who is a good genetic match to the patient. Using stem cells from a closely matched relative helps to improve transplant outcomes.

Cord blood stem cells can be collected and stored at the time of birth and either donated to a public cord blood bank for use by an anonymous patient in need or stored in a private bank for potential use within the family. Cord Blood Registry® (CBR®) is the largest and most experienced family cord blood bank. We offer cord blood collection, processing and storage services to families who wish to save their children's cord blood at the time of birth for the family's potential use in the future. By saving cord blood at the time of birth, families can ensure that they have access to this important resource should anyone in the immediate family ever require a HSC transplant. Many patients with hematological conditions, including MDS, have no family history of the condition, and therefore banking at the time of birth provides a "safety-net" for families. In fact, current estimates suggest that, over an individual's lifetime, the odds of requiring

an HSC transplant are 1 in 217. When stored under the proper conditions, cord blood stem cells are expected to last indefinitely, providing a long-term resource for families' potential use.

As part of CBR's commitment to families in need, our Newborn Possibilities Program® offers cord blood processing and 5 years of storage at no cost for families in which a parent or full sibling of the newborn is diagnosed with a condition for which HSC transplant may be a treatment option. This program provides families with access to a potential source of valuable stem cells, should a transplant be required. To date, CBR has enrolled more than 8,000 families into the Newborn Possibilities Program. Though MDS is typically diagnosed in individuals past child bearing age, families with MDS are eligible to apply for the program. In addition, CBR has Genetic Counselors on staff to help families make informed choices about their newborn stem cell banking options and to answer questions regarding medical indications for cord blood banking.

*For more information about Cord Blood Registry or the Newborn Possibilities Program please call us at 1-888-cordblood or visit [www.cordblood.com](http://www.cordblood.com).*

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### MDS MANAGER

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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 response to treatment.



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# MDS CENTERS OF EXCELLENCE

Our MDS Centers of Excellence are institutions that meet the highest standards for diagnosis, treatment and patient care. These centers help patients seeking first or second opinions and/or additional treatment options from experts in MDS. We currently have 72 Centers in the United States and 112 Centers in countries around the world. Our MDS Centers can be viewed here:

<https://www.mds-foundation.org/mds-centers-of-excellence>



## BENEFITS OF MEMBERSHIP:

- MDSF CoEs form the referral base for the patients who contact the Foundation daily.
- MDSF CoEs are proudly recognized on the Foundation website, within our printed newsletters, and through our various social media platforms.
- MDSF CoEs are offered reduced registration rates at our Int'l Symposia and discounted subscription rates to *Leukemia Research*.
- MDSF CoEs have full access to MDSF educational resources for distribution to your patients.
- In addition, along with your \$500 CoE renewal payment, your annual MDSF Professional Membership dues are waived. MDSF Professional Members are also listed, by name, on our website and in our printed newsletters.
- The work of your institution can be shared with our patient and professional contacts via our website and/or our social media channels. We can spread the word of your clinical trials, research projects, etc.

Would you like your treatment center to become part of the referral system for MDS patients and be designated as a Center of Excellence?

## To be recognized as a Center of Excellence, an institution must have the following:

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

Please contact the Foundation for further information and an application form for your center.

The following centers have qualified as MDS Centers of Excellence:

## UNITED STATES

### ALABAMA

University of Alabama at Birmingham  
Birmingham Comprehensive Cancer Center  
Birmingham, Alabama  
*Kimo Bachiashvili, MD*

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Phoenix, Arizona  
*Cecilia Arana Yi, MD/Lisa Sproat, MD*  
The University of Arizona Cancer Center  
Tucson, Arizona  
*Ravi Krishnadasan, MD, FACP*

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UCLA School of Medicine  
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City of Hope National Medical Center  
Duarte, California  
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Moores Cancer Center-UC San Diego Health  
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*Peter Curtin, MD*  
Stanford University Medical Center  
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Chinese Academy of Medical Sciences  
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# WE NEED YOU

## HOW YOU CAN HELP

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- 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.

### DONATE TO SUPPORT OUR MISSION\*

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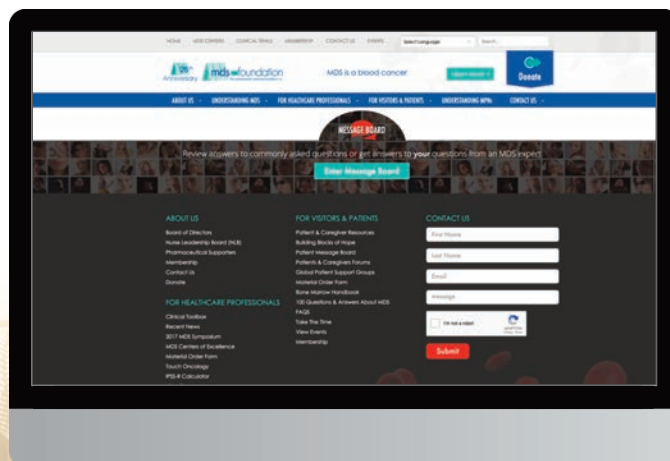
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- Volunteers are vital to our mission.

**\*PLEASE VISIT**

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

to discover how can you join,  
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## THINKING OF JOINING THE MDS FOUNDATION AS A PROFESSIONAL MEMBER OR CHANGING THE FUTURE OF MDS MEMBER?

To join the MDS Foundation and help us fulfill our mission of moving closer to a cure for MDS, please visit our website at <http://www.mds-foundation.org/professional-annual-membership-application>.

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To register by phone or for any questions, call or email Janice Butchko at

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Many patients and caregivers have never met another person diagnosed with MDS until they connected with them at one of our forums. If you've never attended one, you won't want to miss this opportunity to meet others and to learn more about MDS, current treatments, and emerging therapies from leading experts. Not only will you find answers, support and hope for MDS but you will learn tips and strategies for patients and caregivers **LIVING** with MDS.

**PLEASE MAKE SURE TO REGULARLY CHECK OUR WEBSITE  
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### U.S. FDA APPROVES REBLOZYL® (LUSPATERCEPT-AAMT), THE FIRST AND ONLY ERYTHROID MATURATION AGENT, TO TREAT ANEMIA IN ADULTS WITH LOWER-RISK MDS

PRINCETON, NJ & CAMBRIDGE, MA, APRIL 3, 2020 – The FDA approval marks the second indication for Reblozyl and the first new treatment option in over a decade for patients with MDS who require red blood cell (RBC) transfusions and have failed an erythropoiesis stimulating agent.

Reblozyl regulates late-stage RBC maturation to relieve patients from the burden of regular RBC transfusions.

Bristol Myers Squibb (NYSE: BMY) and Acceleron Pharma Inc. (NASDAQ: XLRN) today announced the U.S. Food and Drug Administration (FDA) has approved Reblozyl® (luspatercept-aamt), the first and only erythroid maturation agent (EMA), for the treatment of anemia failing an erythropoiesis stimulating agent and requiring 2 or more red blood cell (RBC) units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T). Reblozyl is not indicated for use as a substitute for RBC transfusions in patients who require immediate correction of anemia.<sup>1</sup>

“In clinical trials, Reblozyl has shown to have significant benefit for the treatment of anemia in patients with myelodysplastic syndromes who have ring sideroblasts,” said Guillermo Garcia-Manero, MD, professor and chief of Section of Myelodysplastic Syndromes, Department of Leukemia, University of Texas MD Anderson Cancer Center. “Anemia is a serious consequence of MDS, requiring the majority of these patients to receive regular red blood cell transfusions, which can lead to additional complications, such as iron

overload, transfusion site reactions and infections. In our current environment, we are reminded of the significant burden frequent blood transfusions can have on individuals and the healthcare system.”

“Today’s approval of Reblozyl is an important milestone for a majority of patients with myelodysplastic syndromes who have limited treatment options to address anemia associated with their disease. It also demonstrates our continued commitment to develop innovative products that improve the lives of patients living with serious diseases,” said Diane McDowell, MD., vice president, Hematology Global Medical Affairs, Bristol Myers Squibb. “We are looking forward to making Reblozyl immediately available for this patient population.”

The FDA approval in MDS is based on results from the pivotal Phase 3 MEDALIST trial and marks the second indication for Reblozyl, which received its first approval in November 2019 for the treatment of anemia in adults with beta thalassemia who require regular RBC transfusions.<sup>1</sup>

“We’re excited that Reblozyl has been approved to help even more patients in need of treatment options,” said Habib Dable, President and Chief Executive Officer, Acceleron. “We are enormously grateful to the patients, families and caregivers who participated in and supported the Reblozyl clinical trials, and to the researchers at Acceleron and beyond who, more than a decade ago, began this important quest to address patients’ chronic anemias.”

#### ABOUT MEDALIST

The approval of Reblozyl was based on the findings of MEDALIST, a Phase 3, randomized, double blind, placebo-controlled, multi-center study evaluating the efficacy and safety of Reblozyl in patients with IPSS-R-defined very low-, low- and intermediate-risk non-del(5q) myelodysplastic syndromes (MDS) with ring sideroblasts. All patients were red blood cell (RBC) transfusion-dependent and were either refractory or intolerant to prior erythropoiesis-

stimulating agent (ESA) therapy or were ESA naïve and unlikely to respond due to endogenous serum erythropoietin  $\geq 200$  U/L, and had no prior treatment with disease modifying agents.

In the trial, a significantly greater proportion of patients receiving Reblozyl achieved independence from RBC transfusions for at least eight weeks during the first 24 weeks of the trial compared with those receiving placebo, meeting the study’s primary endpoint.<sup>2</sup> Additionally, a significantly greater proportion of patients receiving Reblozyl vs. placebo achieved at least 12 weeks of independence from transfusions within the first 24 and 48 weeks of the study.<sup>2</sup>

The majority of treatment-emergent adverse events (TEAEs) in the trial were Grade 1–2. Grade 3 or 4 treatment-emergent adverse events were reported in 42.5% of patients who received Reblozyl and 44.7% of patients who received placebo. The most common (>10%) all-grade adverse reactions included fatigue, musculoskeletal pain, dizziness, diarrhea, dyspnea, nausea, hypersensitivity reactions, headache, and upper respiratory tract infection.<sup>2</sup>

Results from MEDALIST were published in the New England Journal of Medicine in January 2020.

#### ABOUT REBLOZYL®

Reblozyl, the first and only erythroid maturation agent, promotes late-stage red blood cell maturation in animal models.<sup>1</sup> Bristol Myers Squibb and Acceleron are jointly developing Reblozyl as part of a global collaboration. Reblozyl is currently approved in the U.S. for the treatment of:

- anemia in adult patients with beta thalassemia who require regular red blood cell transfusions, and
- anemia failing an erythropoiesis stimulating agent and requiring 2 or more red blood cell units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic syn-

dromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T).

Reblozyl is not indicated for use as a substitute for red blood cell transfusions in patients who require immediate correction of anemia.

#### **Indication**

REBLOZYL is indicated for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions

REBLOZYL is indicated for the treatment of anemia failing an erythropoiesis stimulating agent and requiring 2 or more red blood cell units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T)

REBLOZYL is not indicated for use as a substitute for RBC transfusions in patients who require immediate correction of anemia.

### **COVID-19: IMMEDIATE RELIEF AVAILABLE**

## **BRISTOL MYERS SQUIBB EXPANDS PATIENT SUPPORT PROGRAMS TO HELP NEWLY UNINSURED PATIENTS IN THE U.S.**

PRINCETON, NJ, APRIL 7, 2020 –

Bristol Myers Squibb (NYSE: BMY) today announced an expansion of its existing patient support programs to help eligible unemployed patients in the U.S. who have lost their health insurance due to the COVID-19 pandemic.

The expanded program offers access to any branded Bristol Myers Squibb medicine for free, including some of its most widely prescribed products, as well as those prescribed via telehealth services.

The program features:

- Immediate access for patients who have lost their employment and health insurance;
- A simple, single point of entry;
- Streamlined enrollment process; and
- Vouchers to assist with continuity of care for several self-administered BMS medicines, for eligible patients

All Bristol Myers Squibb patient support programs, as well as, additional eligibility requirements, can be reached by calling (800) 721-8909 or by visiting [BMS.com](https://www.bms.com).

“The COVID-19 pandemic has created unprecedented financial challenges for patients and families, adding considerable new stress to the millions of Americans who have lost their jobs and health insurance,” said Giovanni Caforio, MD, chairman and chief executive officer, Bristol Myers Squibb. “As more patients face difficult decisions in their daily lives, it is important to continue their treatments.”

#### **Bristol Myers Squibb's Response to COVID-19**

Bristol Myers Squibb recognizes this is a challenging time for everyone. The company will continue to take all necessary actions to promote public health and carry out its mission of providing life-saving medicines to the patients who depend on us. Please visit [BMS.com](https://www.bms.com) to learn more about our actions to date.

## **ASTEX PHARMACEUTICALS ANNOUNCES U.S. FOOD AND DRUG ADMINISTRATION (FDA) ACCEPTANCE FOR REVIEW OF AN NDA FOR THE COMBINATION ORAL HYPOMETHYLATING AGENT CEDAZURIDINE AND DECITABINE (ASTX727 OR ORAL C-DEC), FOR THE TREAT- MENT OF MDS AND CMML**

PLEASANTON, CA, FEB 11, 2020 –

Astex Pharmaceuticals announces U.S. Food and Drug Administration (FDA) acceptance for review of an NDA for the combination oral hypomethylating agent cedazuridine and decitabine (ASTX727 or oral C-DEC), for the treatment of MDS and CMML

- NDA is supported by data from the phase 3 ASCERTAIN study of oral C-DEC in adults with intermediate- and high-risk myelodysplastic syndromes (MDS) including chronic myelomonocytic leukemia (CMML)
- FDA designated the application for Priority Review
- Potential for oral C-DEC to become first approved orally administered hypomethylating agent for MDS and CMML in the U.S.

Astex Pharmaceuticals, Inc., a wholly owned subsidiary of Otsuka Pharmaceutical Co. Ltd., based in Japan, today announced that the U.S. FDA has accepted for Priority Review its NDA for oral C-DEC (cedazuridine and decitabine) as a treatment for adults with previously untreated intermediate- and high-risk MDS including CMML. The NDA submission is based on data from the ASCERTAIN phase 3 study which evaluated the 5-day decitabine exposure equivalence of oral C-DEC and IV decitabine.

“We are very pleased that the FDA has accepted our NDA for Priority Review,” said Dr Mohammad Azab, MD, president & chief medical officer of Astex Pharmaceuticals, Inc. “Subject to FDA review and regulatory approval, oral C-DEC may offer a new option for patients with MDS and CMML that saves them the burden of 5-day IV infusions every month during their treatment period. We are grateful to all the patients, investigators and other healthcare providers, and partner research and manufacturing organizations, who contributed to the clinical development program of oral C-DEC.”

The FDA grants Priority Review to applications for drugs that, if approved, would provide significant improvements in

the safety and effectiveness of the treatment, diagnosis or prevention of serious conditions. The Priority Review designation means FDA's goal is to take action on an NDA application within six months (compared to the ten months under standard review).

Oral C-DEC is an investigational compound and is not currently approved in any country.

Astex's parent company, Otsuka Pharmaceutical Co., Ltd., and Taiho Pharmaceutical Co., Ltd. previously announced that, subject to regulatory approvals, commercialization of oral C-DEC in the U.S. and Canada will be conducted by Taiho Oncology, Inc. and Taiho Pharma Canada, Inc. respectively. Astex, Otsuka and Taiho are all members of the Otsuka group of companies.

#### **ABOUT C-DEC (CEDAZURIDINE 100 MG AND DECITABINE 35 MG) FIXED-DOSE COMBINATION**

C-DEC is a novel, orally administered fixed dose combination of cedazuridine, an inhibitor of cytidine deaminase,<sup>1</sup> with the anti-cancer DNA hypomethylating agent, decitabine.<sup>2</sup> By inhibiting cytidine deaminase in the gut and the liver, C-DEC is designed to allow for oral delivery of the approved DNA hypomethylating agent, decitabine, at exposures which emulate exposures achieved with the approved intravenous form of decitabine administered over 5 days.<sup>3</sup>

C-DEC has been evaluated in a phase 1/2 pharmacokinetics-guided dose escalation and dose confirmation study in patients with MDS and CMML (see <https://www.clinicaltrials.gov/NCT02103478>) and a pivotal phase 3 study (ASCERTAIN) (see <https://www.clinicaltrials.gov/NCT03306264>) conducted at investigator sites in the US and Canada and designed to confirm the results from the phase 1/2 study. The phase 3 study is now being extended to include patients with acute myeloid leukemia (AML) unsuitable to receive intensive induction chemotherapy.

In September 2019 Astex announced that C-DEC had received orphan drug

designation for the treatment of MDS and CMML from the U.S. FDA.

The concept of using cedazuridine to block the action of cytidine deaminase is also being evaluated in a low dose formulation of cedazuridine and decitabine for the treatment of lower risk MDS (see <https://www.clinicaltrials.gov/NCT03502668>).

#### **ABOUT THE PHASE 3 ASCERTAIN STUDY**

The study was designed as a randomized crossover study comparing oral C-DEC (cedazuridine 100 mg and decitabine 35 mg fixed-dose combination tablet given once daily for 5 days on a 28-day cycle) to IV decitabine (20 mg/m<sup>2</sup> administered as a daily, 1-hour IV infusion for 5 days on a 28-day cycle) in the first 2 cycles with patients continuing to receive oral C-DEC from Cycle 3 onwards. The data from the ASCERTAIN study was presented at the American Society of Hematology (ASH) Meeting in Orlando, Florida in December 2019 by Dr Guillermo Garcia-Manero, MD, professor and chief of section of myelodysplastic syndromes, Department of Leukemia at The University of Texas MD Anderson Cancer Center, on behalf of the study investigators.<sup>4</sup> The data demonstrated that the ASCERTAIN study met the primary endpoint of total 5-Day decitabine Area-Under-The-Curve (AUC) equivalence of oral C-DEC and IV decitabine. Safety findings from the study were consistent with those anticipated with IV decitabine, with no significant differences in the incidence of most common adverse events between oral C-DEC and IV decitabine in the first 2 randomized cycles. The most common adverse events of any grade >20% regardless of causality in patients in the first 2 randomized cycles who received oral C-DEC were thrombocytopenia (43.8%), neutropenia (35.4%), anemia (36.9%), and fatigue (23.8%). The ASH presentation can be downloaded from the Astex website at <https://astx.com/media-center/presentations-andpublications/ASTX727> ASCERTAIN Presentation - ASH - December 2019.

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## IN YOUR OWN WORDS

### TESTIMONIALS

"Amazing! The website is full of **VALUABLE INFORMATION IN VARIOUS FORMS** for all types of viewers. The information package that was sent home is full of wonderful books that will help my mother learn more about MDS. Definitely exceeded my expectations!!! Thank you so much. It's nice to see an excellent foundation." – Diane S.

"Recently diagnosed with so much to learn, especially about living with it. This organization seems to **OFFER KNOWLEDGE AND SUPPORT**, which I for one, need. While I want to learn as much as I can about the disease, reading about the experiences of others with it I find very comforting." – Pat M.

"I appreciate you speaking with me a few minutes ago, your kind words and prompt email. I have already called Vanderbilt and made an appointment for my father in a few days. **THANK YOU FOR YOUR WORK IN HELPING MDS PATIENTS AND THEIR FAMILIES.** God's blessings!" – Jacqueline A.

"**THANK YOU VERY MUCH** for the work you are doing on behalf of MDS patients, their families, and the larger MDS community." – Joey W.

"I have only recently been diagnosed with MDS, high risk at 5% (although I'm not quite sure what that means – yet). **I AM VERY PLEASED TO HAVE FOUND THIS SITE** and have been reading the message board. I am a 66 year old female. My care will be at Seattle Cancer Alliance. Thank you for making this informative site available!" – Cynthia H.

"Thank you for this great information! I am a PV patient diagnosed in 2012. I appreciate **YOUR TIME AND DEDICATION IN SUPPORT FOR ALL OF US** with these varied diagnoses." – Mary G.

"I wish to inform you that I have well received the book Building Blocks of Hope by the post. Thank you very much. I have started the reading and am sure the **INFORMATION IT CONTAINS WILL BE OF GREAT USE TO UNDERSTAND MDS.**" – Linda C.

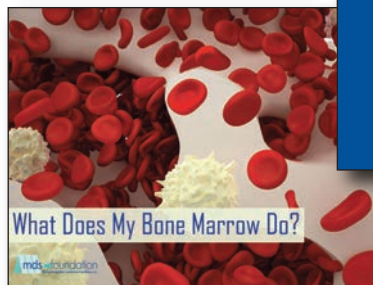
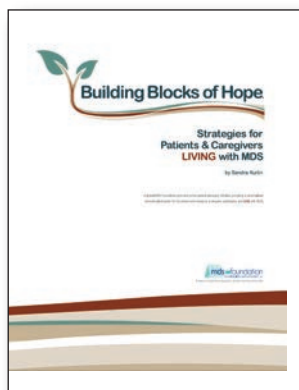
"The Postie delivered your generous parcel today to Perth containing bracelets and books with so much information. In our journey, **YOU ARE THE ONLY PLACE THAT HAS GIVEN US TRUE INFORMATION.** It is great to have a book that you can pick up and put down and so slowly digest the heavy subject." – Kerry J.

**"I am in awe of everything the MDS Foundation is doing. Bless you all for the work you do and the comfort you give."**  
Janice C.

**"The information they share is invaluable and I will call Audrey Hassan whenever I have a question — she is fantastic at research and always has answers for me! I don't feel so alone now. Thank you!"** Jane B.



The Building Blocks of Hope Handbook provides strategies for patients and caregivers living with MDS. This program is designed to give patients and caregivers the in depth information that they are looking for and to allow them to take an active part in their MDS journey. The BBoH is available in several languages. Call 1-800-MDS-0839 for your FREE copy today.



## FIND THE TRUSTED RESOURCES YOU NEED... *You or someone you know has been diagnosed with MDS*



Hearing the words Myelodysplastic Syndromes or MDS can be frightening. The diagnosis of MDS is often unexpected and filled with both immediate and long-term challenges. You probably have many questions. Have you accessed your complete set of tools to prepare, participate, and **LIVE** with MDS?

**Dealing with MDS can be very difficult, but it helps to have helpful resources that are reliable and that you can trust.**

To order your FREE copy of our resources available in multiple languages, please visit our website:

**<https://www.mds-foundation.org/material-order-form-4/>**

## PATIENT STORIES

### OUR PATIENT STORIES

#### MY JOURNEY TO A HAPLOIDENTICAL STEM CELL TRANSPLANT

**TOM HAYGOOD**

*Hendersonville, Tennessee*

I have what?? Myelodysplastic Syndrome?? That's how my journey began six years ago at age 62. It was the fall of 2013 when the common symptoms of MDS started. Fatigue and joint pain became a common occurrence in my everyday life. After a visit to my family doctor, blood tests were run and the results indicated some abnormalities. I was referred to a local hematologist who ordered additional blood tests along with a bone marrow biopsy and CT scan. With those results in hand, I was diagnosed with MDS. Since I'd never heard of the disease, the MDS Foundation and the Leukemia & Lymphoma Society were excellent resources to learn more about MDS. I also joined an MDS support group on Facebook.

I live in Hendersonville, Tennessee, a suburb of Nashville, and was fortunate that I was able to receive all my treatment close to home in Nashville. In January 2014, I was referred to Tennessee Oncology at the Sarah Cannon Center for Blood Cancer at Centennial Medical Center. The Center is named after patient Sarah Cannon who was better known as Minnie Pearl of the Grand Ole Opry! She offered the use of her given name and financial support to promote cancer research and patient education.

My subtype of MDS was RAEB. I was on "watch & wait" for 8 months. When my blood counts became lower and another bone marrow biopsy showed an increase in blasts, I began treatment with Vidaza in August 2014. I went 7 days a month, M-F and M-Tu and received 3 subcutaneous injections in my stomach. Other than irritation at the shot sites and fatigue, I had minimal side effects, but I did retire in September since my job required traveling and my immune system was very weak.



After 19 months of shots, I had a port implanted in March 2016 and began receiving Vidaza infusions on the same 7 day schedule. In October 2016, I walked my daughter down the aisle at her wedding in Denver! I changed to a 5 day schedule of infusions in January 2017.

In June 2017, after 32 rounds of Vidaza, my counts did not recover and a bone marrow biopsy indicated 12% blasts. I was referred to Dr. Carlos Bachier at the Blood and Marrow Transplant Clinic at Sarah Cannon Cancer Center. I was evaluated with a series of tests and determined to be a good candidate for a stem cell transplant. Since there was no donor match from the international registry and my siblings were not suitable donors due to health issues, the decision was made to have a haploidentical SCT. I have two daughters so they were both evaluated as potential donors. My younger daughter Lisa, 32, was chosen as my donor since she had never been pregnant, and my older daughter had given birth to our three awesome grandchildren.

I was admitted to the Immunosuppression Unit at Sarah Cannon Cancer Center on August 21, 2017, which was the date of the solar eclipse. That seemed appropriate since we anticipated some dark days



ahead. I began an intense round of 7-3 induction chemo to prepare my immune system for my upcoming SCT. The chemo was administered thru a PICC line. My wife kept a daily journal and began a website for me on CaringBridge.org to keep our family and friends updated. I had severe mucositis from the chemo which made it difficult to eat. I knew I would eventually lose my hair, so I decided to shave my head one night. My wife was surprised the next morning to find a strange bald headed man in my room! On days when I felt good enough, I walked laps around the floor with a goal of 2 miles a day. My bone marrow biopsy showed no evidence of



*Tom's donor – his daughter, Lisa*

## OUR PATIENT STORIES

disease, but I had to wait until my ANC count increased before I could be discharged. After 32 days in the hospital, I went home on Sept. 21 for 15 days. During that time, I had a Central Venous Catheter implanted for my upcoming SCT.

On October 6, 2017, I was readmitted to the hospital for 5 days of chemo and one Total Body Irradiation treatment prior to my SCT. Lisa arrived from Denver and began her series of Neupogen injections to stimulate stem cell growth. She received two shots in her stomach for 5 days. She did not have any side effects from the shots and even ran in a 5K event one morning before coming to the hospital for her shots. She posted daily videos on Facebook to encourage others to register as donors on [BetheMatch.org](http://BetheMatch.org). On October 11, she had 580 million stem cells harvested in 7 hours of apheresis. Her only complaint was being bored during the procedure! I consider myself conservative, and it was the family joke that I would change after receiving her liberal stem cells! That didn't happen. They were stem cells, not brain cells!

On October 12, 2017, with my family surrounding me, I had my SCT which took about 30 minutes. Afterwards, I was given another 3 days of chemo and began all the medications for transplant patients. On the 18th day after my transplant, my bone marrow biopsy showed no blasts! I became an impatient patient waiting for my counts to increase. Finally, after 25 days in the hospital, I was discharged on October 30. Since I live within 30-45 minutes of the hospital, I was thrilled that I was allowed to go home instead of staying in a facility near the hospital. It was a real treat to be home for Halloween. My bone marrow biopsy in November showed 100% donor cells and no blasts! I had lots of follow-up appointments and stayed at home most of that winter. Since that time, I have had regular follow-up visits and bone



marrow biopsies. I have had some minor skin and lung GVHD and neuropathy in my feet, but no other complications.

I celebrated my 2nd birthday last October, and I enjoy playing golf with friends 3-4 days a week! I am fortunate that my haploidentical transplant was successful. I have been blessed with great

doctors and nurses, supportive family and friends, and lots of answered prayers in the past six years, and I am thankful for my wonderful wife Gail who has been by my side in sickness and health for 47 years. I hope my journey will be encouraging to others diagnosed with MDS.

## LOOKING FORWARD

**JANET B. WARFIELD, MSN**

*Nashville, Tennessee*

My journey with MDS began in March of 2014. My husband, Bill and I flew to San Diego and then took a beautiful drive through the San Bernardino mountains to Indian Wells. We had tickets to the Indian Wells professional tennis tournament. I was especially excited because we were going to get to see Rafa Nadal play. We arrived on a Friday night exhausted from a long day of travel. I remember not feeling very well but went to dinner and then bed hoping a good nights sleep was all I needed. Bill was up bright and early the next morning. Although we had tickets to see Rafa play that night, Bill went in search of tickets for the day sessions. I realized after he left the hotel room that I literally could not get out of bed. I googled altitude sickness as I was self diagnosing the sudden onset of my fatigue. I slept all day and forced myself up and on the bus to the stadium to meet Bill for the night session to see Rafa play. This was a bucket list moment for me and I wasn't going to miss it.

Upon our return to Nashville, I had an appointment for my annual physical with my general practitioner. I told him about my episode of fatigue while on vacation. He said everything checked out but he would be in touch with lab results from bloodwork. A week or so later I received a letter that my blood was showing signs of anemia and I needed to return for more specific testing for vitamin deficiencies. A few days later, I received a call that changed my life. He said I didn't have a vitamin deficiency and that he was referring me to a blood specialist for further testing. I googled the specialist and my heart almost stopped when I saw he was sending me to an oncologist.

My oncologist couldn't have been nicer or more honest. After additional blood work, a PET scan and a bone marrow



biopsy I was diagnosed with primary MDS. The fear that I experienced at that moment still resonates with me today. I learned that I was young at age 58 to have this rare form of blood cancer. My SF3B1 mutation with ringed sideroblasts put me in the low risk category of MDS which meant that I was not at risk for developing leukemia. It also meant there were currently no treatment options. So I left the oncologist that day in May of 2014 with a diagnosis of bone marrow cancer with a plan to return every 8 weeks for bloodwork. As long as my numbers stayed within a certain range, I would not qualify for any of the available medical treatment protocols for MDS which was a good thing. The best advice he could give me was to "just go live my life".



My life at that time was very busy and blessed. I was a wife to a very special man, mother to a beautiful 24 yr old daughter, Carly, and two precious King Charles Spaniels as well as a daughter to two wonderful aging parents. I was CFO of a nonprofit called Mending Hearts, Inc. which is a residential treatment facility for indigent women. I also had been a competitive tennis player for 20 years.

I needed to learn more about this disease. I found the MDS Foundation online. I requested information and they sent me the Building Blocks of Hope, which I still use as a resource today. I have attended three of their patient forums and participated in both Boston walks. The staff at the MDS Foundation is a group of incredible women who have given me support and hope and I'm so very grateful to each of them. I've tried volunteering for MDS research studies but so far I've not qualified since I've not had any type of treatment. Instead of focusing on my illness, I've been trying to focus on educating myself and connecting with others in the MDS community. I'm so excited that Nashville will be hosting a MDS Awareness Walk in the fall of this year! It's so important that we bring awareness to this devastating disease.

I also searched out alternative treatment options in an attempt to improve my health. I began seeing a doctor of naturopathy in January 2015. I still see her twice a month for lymphatic massage, O2 therapy and bemer mat sessions. She advises me on nutritional supplements that address my suppressed immune system. She also encourages me to eat healthier. I have added Pilates and weight training to my exercise regimen. Are any of these things actually helping my MDS symptoms? I can say that when I adhere to the regimen I have more energy. I have succumbed to just one or two infections a year and my blood levels have remained about the same since 2014. I believe that being

## OUR PATIENT STORIES

proactive about my health has given me a feeling of empowerment and it's been a way for me to actively manage my disease.

I have tried not to change my life dramatically following my diagnosis. I experience some fatigue most days and shortness of breath when walking if the terrain is not flat. I've found that I'm more susceptible to infections. I've gradually learned what I need to do to manage these symptoms. I did quit my job in the summer of 2014. My parents' health was beginning to fail and I wanted to be able to devote more of my time to them. My sweet mom passed in 2016. I miss her so much. My dad celebrated 93 years in October of last year. He has multiple health issues and is now living in a skilled nursing care facility but has the greatest attitude and I treasure every minute I get to spend with him. I am still playing competitive tennis, although not at the same level I

played when first diagnosed. My visits to the oncologist are still just every 8 weeks for bloodwork and for that I'm most grateful.

My husband and daughter have been my rock and they are the reason that each day is a blessing for me. They encourage me to do all that I can but are so supportive when I need to slow down. MDS has had a positive effect on how our family chooses to live our daily lives. MDS was a huge wake up call that life is short and each day is a gift not to be taken for granted. I keep my bucket list close by and am constantly updating it. It keeps me hopeful and focused on looking forward. I still experience the fear that comes with having MDS, but that fear does not keep me from following my doctors orders to "go live my life". I realize I'm one of the luckier ones with a MDS diagnosis and that my prognosis is better than most people in the MDS community.



## THANK YOU TO OUR INDUSTRY PARTNERS FOR THEIR SUPPORT



**A SPECIAL  
THANK YOU**  
for their very generous  
grant in support of  
MDS research

**GABRIELLE'S  
ANGEL FOUNDATION  
FOR CANCER RESEARCH**



## CHASING THE CARROT...

**JILL WHITNEY**

*Redding, California*

My journey with MDS (Myelodysplastic Syndromes) began in December 2007. After playing volleyball with my youngest daughter, I noticed that my forearms were badly bruised. This along with frequent headaches and fatigue prompted me to schedule an appointment for a physical. Little did I know our lives were about to change forever.

We were getting ready to depart on a cruise to Cabo San Lucas, Mexico to celebrate my in-laws' 50th anniversary, but my local hematologist wouldn't allow me to go until a bone marrow biopsy was done. At first, he suspected a vitamin B-12 deficiency. B-12 injections helped for a short time, but then my counts slowly began to decline, especially my platelets.

After six long months and numerous tests, I was finally referred to a hematologist at Stanford University Hospital in Palo Alto, California. My husband and I were told that I had MDS and a bone marrow transplant was the only cure. It was a lot for us to absorb. We somehow managed to hold it together through the appointment, but as soon as we got to our car, we held each other and cried. I was only 47. I couldn't help but wonder if I would ever get to see our youngest daughter graduate from high school or even hold our first grandchild. I couldn't bear the thought of leaving my family.

At the time, I was considered intermediate to high risk and my only sibling was my half-brother, Jim, so a search began through the National Marrow Donor Program. I continued working until we received word that four possible donor matches were found. Our youngest daughter was 14 and Stanford was four hours away from our home in Redding, California. We had a lot of preparation to do before the transplant, so



I left my job as a registered dental assistant and my counts stabilized. We were able to put everything on hold for nearly a year and a half. During this time, our first granddaughter arrived.

I was scheduled to begin the conditioning for the transplant (cytotoxin and busulfan) on May 4, 2010, but due to respiratory symptoms, it was delayed. Having to wait an additional two weeks was disappointing, although I know it was for my own safety. I was admitted to the bone marrow transplant unit at Stanford on May 17th and on May 25th I received the life-saving stem cells from a 47-year old male, German donor. I had a few setbacks that kept me in isolation longer than expected. I spent four days in the ICU due to an E. Coli blood infection and then contracted BK virus. I watched the days

pass by on a giant calendar that showed my daily blood counts and notes of inspiration from the nurses. I had hoped to be discharged in time to spend the 4th of July with my husband, but that day passed. One good thing that came out of my late release, was getting to meet Alex Smith, quarterback for the San Francisco 49er's and several other team members when they paid a surprise visit to the BMT Unit.

On day +44, I was released to an apartment we had rented nearby. At first, my husband took me to the infusion treatment area daily for blood draws and to receive needed transfusions. When he returned to work, I was moved to my parents' home, about 30 minutes from Stanford. Then, I came down with CMV virus and had to remain close to Stanford for an additional 6 weeks. I finally returned to my home in Redding at the end of September 2010.

Eight months following the transplant, we were told that I had relapsed and a second transplant wasn't an option due to congestive heart failure. I was given a prognosis of a few weeks to possibly a few years, if I began treatment. I had not even recovered from the transplant and was now facing chemotherapy. We were devastated. Desperate for help, my husband and I attended an MDS Foundation conference in San Francisco, where Jason Gotlib, MD was a guest



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speaker. My husband approached him after the event and asked if he would accept me as a patient. I started receiving Vidaza (azacitadine) via port-a-cath in February 2011, but my counts had a hard time recovering. For the first few months, I was so sick that I needed help getting out of bed. I was nauseated and had no appetite. My family begged me to eat. At times, I wanted to give up.

I gave myself insulin and Neupogen injections and had to take numerous medications. For 18 months, I required frequent transfusions until a bad reaction caused me to break out in huge welts all over my body. From then on, the platelets had to be HLA matched and packed. Luckily, things started turning around. Our second granddaughter arrived in February 2012. I pushed myself to get better so that I could be present at our youngest daughter's high school graduation and our middle daughter's wedding a few months later. My family kept dangling these carrots out in front of me so that I would keep fighting.



I recently completed my 72nd cycle of Vidaza (azacitadine) and it has now been nine years since my relapse. On May 25, 2020, I will CELLebrate the tenth anniversary of my stem cell transplant (re-birthday). I tolerate chemotherapy pretty well now and take medication to mitigate the side effects. When it is no longer effective, we will have to look at other options, but I am so fortunate to have made it this far.

Unable to work due to ongoing treatments and fluctuating blood counts, I

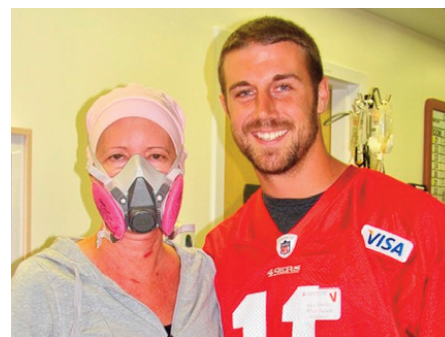


searched for a way to give back and keep my mind off my illness. I volunteered in the Cancer Resource Center at our local hospital for a while and then I came up with an idea where I could work from home. I started Signs of Hope and I have now created over a hundred inspirational signs to help others going through a difficult time. Some I have donated to our local hospital and to the oncology center where I receive treatment. I also started an MDS support group in my community with the assistance of the MDS Foundation.

I am forever grateful for the incredible gift of life from my donor, Michael, and for the care I have received from the outstanding doctors, nurses and support staff at Mercy Medical Center and Stanford, including Dr. Gotlib and transplant specialist, Dr. Wen- Kai Weng. I

also would not be here today without my amazing family. Their love and encouragement is what has kept me pushing forward. My husband, Roger, has been my rock. I can't count how many times he has driven me back and forth to Stanford or how many hours he has spent sitting with me in doctor's offices. He cooked, cleaned and took care of our youngest daughter and me. I know it wasn't an easy job. My parents, Rick and Lynne, and our eldest daughter, Amber, were my caregivers when my husband was working and daughters, Candace and Kaylynn, helped in any way they could. We also had the support of extended family and friends.

I'm not done living yet. I have too much to look forward to. My husband and I both turn 60 this year and in 2021 we will celebrate our 40th anniversary. I now have five beautiful grandchildren that keep me on my toes and I want to be around to watch them grow up. I have to keep chasing those carrots.



### MY HERO

**SUSAN URBAN**

*Carlsbad, California*

I would like to take this opportunity to tell you about a very special man that was in my life, he was my hero, he was my father. My father was born and raised in Los Angeles by his parents, two Italian immigrants from Italy. My father always wanted to be a firefighter with the Los Angeles City Fire Department. He was a very special man, and always had a desire to help his fellow man. Then World War II broke out so my father enlisted in the United States Navy. He served his country well as a machinist mate 2nd class aboard the USS Bliss (a troop transport). After the war, my father worked odd jobs until he was able to live out his dream of becoming a Los Angeles City Firefighter. Whatever job my father held, he put his heart and soul into it and did the best job that he could. He lived, breathed and ate the Los Angeles City Fire Department. He made many friends on the job and truly believed in "The Brotherhood" that firemen have. While on the job, he worked side jobs such as he was the safety officer when they dynamited Chavez Ravine and built what is now Dodger Stadium. The fire department was also responsible to have a fireman as a safety officer on movie locations and my father worked with the likes of John Wayne and Kirk Douglas. He loved his job. He remained on the Los Angeles City Fire Department for 25 years until his retirement in 1977. It wasn't the job that made him retire, he was tired of all the traffic and smog that comes with living in Los Angeles. It was his time to relax and enjoy his retirement in Carlsbad, CA. He still stayed connected with his "firemen" buddies and would meet monthly with a group of them for breakfast and talk about the job. Once a fireman always a fireman.

Sometime around 2009, I went to visit my father in the hospital and overheard he and my mother speaking about seeking an appointment with an oncologist. The word "oncologist" stuck in my head and I asked him why he had to see an oncologist. My



*Susan and her father, and hero.*

father did not lie very well and he told me "Oh, he does other things besides oncology". I'm thinking to myself "no way". After visiting with my father, I went home and immediately called my brother and told him what dad had said. I told my brother that something wasn't right. After my father got out of the hospital my brother and I went over to his house and confronted him about what I heard him say in the hospital. It was then that he told us that he had MDS and had had it for 4 years already. We asked him why he didn't tell us that he had it and he said "I didn't want people to feel sorry for me".

I started finding out as much as I could about MDS and that is when I found out that there were no MDS support groups in Southern California. I wanted my father to get as much information about the disease and by starting the first Southern California MDS Support Group in Oceanside, I was able to get a lot of great speakers who had some valuable information. I not only wanted to help my father but we had a great bunch of people in our support group who became "my family" and I wanted to help them as well. I couldn't stress enough for the members (including my father) to go to an MDS specialist not just a general hematologist or oncologist. My father had been going to a general oncologist who basically just gave my father over 80 blood transfusions, which gave him iron overload. My brother and I took our dad to the City of Hope and to Moores Cancer Center for second opinions, but by the time we knew of my dad's disease and

were able to get him to these two facilities there was nothing more they could do for him.

My father got progressively worse and his quality of life was nothing. He decided he had had enough and he refused any more treatment.

Then on November 1, 2012, my hero, this man that had been my source of strength for so many years, was taken away from me by this ugly disease, MDS. While running the support group in Oceanside, I organized a blood drive with the San Diego Blood Bank and we had a huge success in getting a lot of blood donated and it was marked to be used only for MDS patients. My only regret is that my father passed away two weeks before we had the blood drive so he could not see the amount of people who so generously donated their blood to help people they did not even know. I was overwhelmed by the amount of Marines that drove by the donation site, saw the sign and stopped to donate blood. It kind of came full circle. Here were men defending our country (as my father did during WWII) and now donating blood to help people they didn't know (like my father helped people he didn't know by putting out fires in Los Angeles). I contacted the Oceanside Fire Department and shared my father's story and that he would be so proud if the fire department participated in the blood drive in some way. They very generously sent a vintage fire truck manned with firefighters to the blood donation site, and stayed for the entire time speaking with everyone and letting them take a look at the fire truck. I was very touched by their generosity.

I am now in charge of a new MDS Support Group and we meet at St. Patrick Catholic Church in Carlsbad, CA. I also planned another blood drive on November 16, 2019 and invited the Carlsbad Fire Department. The public is grateful for all that firefighters do and it drew a lot of people to donate for those deserving people who need it to live. It was icing on the cake as some Carlsbad firemen also came and donated blood in honor of their fellow firefighter, my father (Remo Tersolo), my HERO. The Brotherhood...

## OUR CAREGIVER STORIES

### A LITTLE DIFFERENT STORY

**LORI NELSON**

*Burnsville, Minnesota*

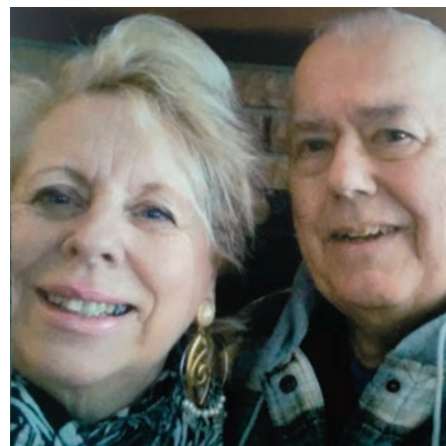
Two years ago, my husband Curt (74 years old) was told that he had MDS. What was this new chapter in our lives?? Did we know anyone in our circle of friends who we could identify with? Did anyone have knowledge or tips for us?

Turning to the internet, I found the MDS Foundation and reached out to them. Wonderful material resources soon arrived in the mail; however, we came to find out that there weren't any MDS Support Groups in our region of Minnesota. The MDS Foundation, Patient Liaison, Audrey Hassan, suggested "YOU could start one." ME? Maybe we could! What would we need to facilitate a meeting? Audrey suggested that we first find a meeting place and that the MDS Foundation could help provide startup funds that could be used to buy refreshments. We placed a few meeting notices in the local newspapers,

the foundation posted information about our group on their website and on their social media platforms and sent us a box of great materials to distribute, and I bought cookies. And yes...they came.

Our group of 15 meets 4 times a year at the library community room. We share treatments, favorite medical teams, and tips to endure transfusions and treatments. One thing we have in common is that we know no one, outside of the support group, who has MDS. It can be a lonely walk with MDS so it's so important to know that you are NOT alone.

At this time, my husband's blood counts are high enough to not require any treatment. We are privileged to know this wonderful group of caring and supportive people.



***If you live in a state without an MDS Support Group just buy some cookies and find a community space...and they will come!!!***

**MINNESOTA MDS SUPPORT GROUP:** The Minnesota MDS Patient Support Group meets quarterly 3:30 PM at Burnhaven Library Main Meeting Room, 1101 County Road 42 W, Burnsville, MN 55306 (corner of Burnhaven and Co. 42). Contact Group Leader, Lori Nelson at 952-892-3659 or email [curtlori45@yahoo.com](mailto:curtlori45@yahoo.com) for upcoming events.

## NEW MDS SUPPORT GROUP TOOLKIT NOW AVAILABLE

### ONE OF MDSF'S GOALS IS TO EXPAND THE NUMBER OF SUPPORT GROUPS WORLDWIDE

*We are always on the lookout for people who are willing to start a group, offer some support in their area, and widen our support services to include more individuals affected by MDS.*

We want to make it as easy as possible for volunteers to create and run support groups to ensure they can provide continuous comfort to those utilizing them.

We have created a **Support Group Leader Toolkit** that will provide guidance on how to run the group, suggestions for discussion topics, useful tools like sample letters and forms, and various other resources to help the meetings run smoothly.

***If you are interested in starting a support group of your own, please contact Audrey Hassan at 1-800-MDS-0839 Ext. 210 or email [ahassan@mds-foundation.org](mailto:ahassan@mds-foundation.org).***



### MDS SUPPORT GROUP LEADER'S TOOLKIT

*A Helpful Guide for Support Group Leaders*

The Toolkit, along with downloadable sample forms can be viewed online.

**mds foundation**  
The Myelodysplastic Syndromes Foundation, Inc.  
[www.mds-foundation.org](http://www.mds-foundation.org)

## AML CORNER

### AML PATIENT STORIES

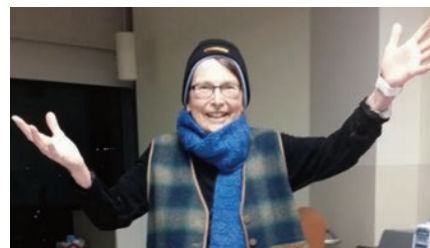
## YOU AND AML: AN ANIMATED PATIENT'S GUIDE TO ACUTE MYELOID LEUKEMIA

This resource is intended for patients with acute myeloid leukemia (AML). You will find expert advice about AML, AML with myelodysplasia-related changes (AML-MRC) and treatment-related AML (tAML) to help you discuss key issues with your healthcare provider and make important decisions related to management and treatment.

### "YOU AND AML" CONTAINS 4 LEARNING MODULES:

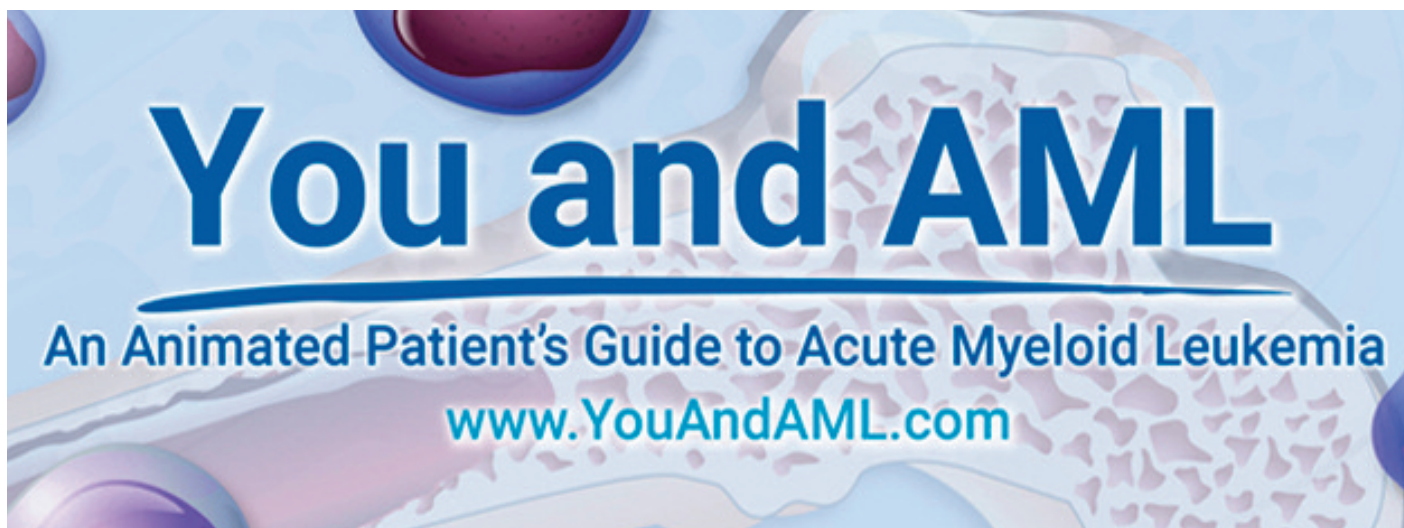
- Understanding AML
- Diagnosing AML, AML-MRC and tAML
- Understanding AML-MRC and tAML
- Treatment of AML

Each module contains easy-to-understand animations with audio narration, video explanations by AML experts, patient interviews, and illustrated slide shows.



**EACH PATIENT HAS THEIR  
OWN UNIQUE STORY**

**MEET AML PATIENT  
VALERIE FONS**



## SURVIVOR

### VALERIE FONS

*Washington Island, Wisconsin*

*God on high, hear my prayer,  
In my need, you have always  
been there.*

*He is young, he's afraid, let him rest,  
heaven blest.*

*Bring him peace. Bring him joy.*

*He is young. He is only a boy.*

*Let him live.*

*You can take. You can give. Let him be.*

*Bring Him Home, Les Miserables*



Surrounded by the Atlantic Ocean and the Caribbean Sea is the Island of Hispaniola and the town of Puerto Plata. When my canoeing partner and I landed at this seaport town, we had already traveled nine thousand miles in our solo canoes from the Arctic Ocean enroute to Cape Horn, Chile. I sought the cool peace of sanctuary and walked down a street paved in dirt, bordered by open sewer ditches to a stone building with the sign of a cross.

On the steps of the St. Philip Cathedral, women are holding babies. Lamé, and diseased, with bleeding sores on his leg, a man sat on the steps with his open palm propped on his bent knee.

Small children stand with distended bellies, not playing but quiet, watching. All have eyes of longing. Each hand is outstretched in need. I walk between the people, through the open wooden doors, onto the marble aisle, making my way to the altar where I kneel and thank God for our progress to this island.

The journey has not been easy. There were landslides on the Mackenzie River, Northwest Territories, Canada. My partner broke ribs near Ft. Simpson. Inebriated locals chased us at Red Dog. We were frozen to the seats of our canoes in Keweenaw Bay, spooked by alligators in Florida, and paddled nonstop for fifty hours on an open water crossing in the Caribbean. There were ten thousand miles of water still ahead. In the cathedral at Puerto Plata, I breathed deeply. The furrow on my brow that was becoming a characteristic facial expression began to relax, for I had escaped the noon heat and felt that at this moment there was no threat.

When I stood from the prayer rail to leave the church, a woman came behind me and touched my arm. When I turned toward her, I could see her hand open and waiting for help. My spirit was refreshed by a time of prayer, I reached into my pocket and gave her a coin.

When the money touched her palm, she opened her mouth. In the dimly lit church, her jaws became like a cavern of anguish that seemed to swallow all remaining daylight. She had no teeth and her wagging tongue vibrated with the sudden energy of a rallying cry. The woman called her friends. I had been singled out as one who would give. Immediately, a throng of beggars pressed against me, all wanting help. I drew back and escaped into the street, hurrying away, sure that I was not meant to provide for all these people.

**HOW MUCH COULD I GIVE?  
I HAD ANSWERED THAT  
QUESTION WHEN WE BEGAN.  
I WAS GOING TO GIVE  
EVERYTHING I HAD.  
YET, EVERY MOMENT I WAS  
ASKED FOR MORE...  
THE JOURNEY HAS NOT  
BEEN EASY.**

At times, the expedition itself pressed like a throng of beggars. During the thirty-three-month journey, the question was the same; how much could I give? I had answered that question when we began. I was going to give everything I had. Yet, every moment I was asked for more.

Twenty years after reaching Cape Horn, I was diagnosed with ALL. A bone marrow transplant gave me six years before the treatments I had endured induced MDS. I became transfusion dependent. When MDS morphed into AML, doctors recommended salvage chemotherapy. In 2018, with 40% leukemia blasts in my marrow, I traveled to the University of Washington Hospital for a clinical trial named G-Clam Long Arm, endured a radioactive isotope clinical trial as the first woman in the trial following rats, dogs and three men. Full body radiation was followed by an unrelated donor stem cell transplant.

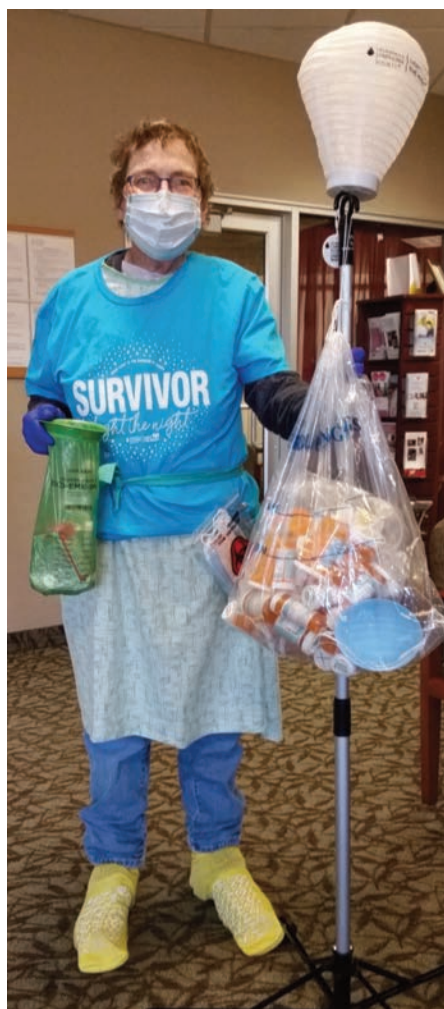
The journey has not been easy. The beggars I encountered in the church during my paddling journey? I am one of them now. I have eyes of longing. I am on the take with my palm open, hand out, arm raised, and life at stake receiving the sacrifices of bone marrow and stem cell donors. I beg my donor cells to go easy on the attack mode within my system as my graph vs. host

disease blooms past acute to severe and chronic. Dr. Sandeep Jain, the ocular GVHD specialist at the University of Illinois, Chicago, evaluated my Ocular Surface Disease Index score at 83.3 with a symptom intensity of 6/10 termed "Distressing/Miserable." My tear production is zero, injured beyond repair. I invited the Wisconsin Department of Human Services, Office of the Blind and Vision Impaired when I was at the mercy of state services and contributions of adaptive equipment so I could know what time it is. In a world of pain and blur, I learned to close my eyes and listen more intently. Asking my children to read to me. Drive me to an appointment because I am no longer able. Hoping doctors do their homework. Panhandling for persistent, dedicated researchers. Scrounging for clinical trials. Entreating schedulers to find an appointment for me with the next recommended expert. Seeking Angel Flights and the kindness of volunteers, friends, and family to deliver me to medical centers. Becoming a vagrant, waiting for the lab tech to go easy with the stick and promise not to fish with the needle. "Draw blood the first try," I tell them. "Use heat compress



*Valerie checking into University of Washington Hospital for salvage chemotherapy with GClam LongArm clinical trial.*

and tourniquet," I suggest to the phlebotomist since I know my body best. When my port, double lumen Hickman, and PICs are removed, leaving one, reliable soldier vein, I strive to place my arm in the best possible position to help the clinical pierce my skin and draw enough to fill the red, green, purple, gold, and/or blue cap tubes with doctor's orders. I rattle the cage when a test resultant is not forthcoming. I demand another provider when the lumbar puncture goes bad with my body curved in a fetal position, facing the wall on the procedure table as the first assigned provider pokes multiple stabs without finding the open space between vertebra. I ask the nurse to hold my hand as the doctor begins pressing my pelvis to find where to place the needle for a bone marrow biopsy and aspiration. I beg my health insurance not to run out. When I am in the hospital, I beg to return home. I hope for a twenty-dollar bill to surface in my wallet, or a credit card that has not maxed its limit to meet another co-pay or call a specialty pharmacy to extend credit. I track down social workers for food, gas gift cards, cab vouchers to still the tide of my evaporating finances. I rustle grants, heat credits, food stamps, and loans, tapping the equity of my house and car to pay the bills. I sit across from hospital financial aid officers making applications for reduced or waived payments on my bills. Instead of a princess sticker as reward following procedures, I squirrel away juice, cheese, and snacks to bolster my food security. During infusion procedures, I ask for double the snacks I can eat and stuff my purse for treats to munch on later. I seek bargains on clothes and shoes for me and my children at rummage and garage sales. I bargain with myself to get out of bed, beat the incessant fatigue and flex my steroid melted muscles, stripped bones, frayed joints, and fragile, thin skin compromised by treatment. I beg for my



*Halloween at the hospital. Valerie dressed with Survivor t-shirt, carrying a bag of her empty medicine bottles, using a barf bag to collect candy*

voice and body to be counted rather than submitting as a data point, guinea pig, or lab rat. I beg the pharmacist to tell me the side effects of each new medication even while the doctor orders the drug, upholds protocol, and cautions the downside of not swallowing the elixir. I beg for peace and understanding to accept my new normal even as I recognize the dynamics of apogee – the developmental culmination of the moon orbit in the position farthest from the earth – seeking orientation in my position as cancer patient in territory I had never imagined or asked for while struggling to find baring and purpose for my altered life farthest from where and what I knew myself to be before cancer.

Grace is abundant. Without asking, I am showered with cards, prayers, cancer caps, wig, lap robes, smiles, encouragement, demonstration and the practice of courage in the world of cancer survivors, caregivers, and donors. MDS Foundation, Be the Match Foundation, American Cancer, Blood and Marrow Transplant Info Network, and Leukemia/Lymphoma Societies are sources for newsletters, webinars, conferences, websites stocked with information, free counseling, referrals, grants, and more. Do people bearing gifts, institutional staff and volunteers



**Valerie's bone marrow biopsy enroute to lab for testing**

realize their work and kindness are critical for so many of us?

People tell me they do not like asking for help, and prefer independence. I remind myself and other advocates of pulling oneself up by bootstraps that we are conceived dependent in our mother's wombs. We are born dependent. Surviving cancer is a great equalizer, dependent on the kindness of others even when we hide in bed with covers pulled over our heads, exhorting the world to go away, while grappling with the necessity of support teams and wondering who amongst family and friends are willing to come alongside. Sometimes I notice a new best friend in the patient beside me. We are two bald heads speaking cancer language.

I cry out to God for another day of being alive. I beseech thee, O Lord, for mercy. Victim is not the word I use or the place I want to be. In the midst of my circumstance, gratitude is bedrock and overarching privilege on the journey to well-being. I advocate for wholeness and



Valerie and her son Joshua at Leukemia/Lymphoma Society Light the Night Walk



*Honorary Black Belt, presented to Valerie by Gary Vernon with a certificate of Taekwondo Rank for loving service to others, lifetime accomplishments and indomitable spirit in the face of great adversity. Signed by Grandmaster Joseph L. Connelly, Seventh Degree Black Belt US Moo Sui Kwan and Gary Vernon, Second Degree Black Belt.*

healing. Begging that I can take up my pallet and walk with the army of survivors moving forward into a new day.

Cancer reminds me of my 21,000-mile canoe journey from the Arctic Ocean to Cape Horn. During my decade of cancer and treatments, the question is the same. How much can I give? I am giving everything I have, even as every moment I am asked for more. And, I am receiving more love, learning, and care, while energized with a rallying cry of thanks!



*Self portrait art therapy at University of Washington Hospital.*



Valerie Fons is a cancer survivor of ALL, MDS, and AML, a bone marrow transplant in 2010, and stem cell transplant in 2018. Diagnosed with MDS in 2016, doctors told Valerie she was dying. In response, she wrote lyrics of affirmation and life set to the melodies of popular show tunes. The American Transplant Games heard Valerie's songs and accepted her as a participant at the 2020 games in the vocal lyrics category. In July, 2020, she will be singing her good marrow songs in Meadowlands, New Jersey. She lives on Washington Island with six adopted children, her husband Joe, and a dog named HOPE.

Do you...

# KNOW AML

## ACUTE MYELOID LEUKEMIA

also called AML, is a type of cancer that affects the stem cells in the bone marrow. It is one of the most common types of leukemia in adults, but can develop in children too.



@KNOW\_AML



#KnowAML



## KNOW AML

Know AML is the first global AML awareness and education initiative. Our goal is to facilitate and improve AML knowledge worldwide and develop community-based initiatives to overcome current and future challenges.

For more information, visit:

[www.know-aml.com](http://www.know-aml.com)

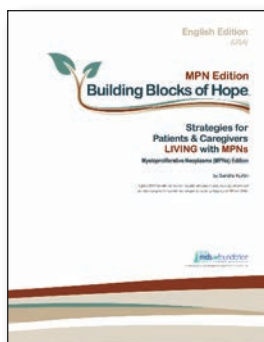
## SIGNS & SYMPTOMS

AML develops quickly, which is why it is termed “acute”, and is often diagnosed at an advanced stage. It is important for everyone to fully understand the signs and symptoms to ensure a diagnosis is made as early as possible.

Here are some of the general signs and symptoms of AML to look out for:

- ✓ WEIGHT LOSS
- ✓ TIREDNESS OR FATIGUE
- ✓ FEVER
- ✓ LOSS OF APPETITE
- ✓ NIGHT SWEATS
- ✓ BLEEDING/BRUISING MORE EASILY





### MPN EDITION – BUILDING BLOCKS OF HOPE RESOURCE

As a continuum of our efforts we have expanded our focus to include education and research into related myeloproliferative neoplasms (MPN), the so called overlap syndromes, especially given their similarity to MDS in their clinical presentation and the mutations that accompany them.

Our new MDS/MPN patient and caregiver resource, Building Blocks of Hope® MPN Editions, was completed to meet the needs of patients who are LIVING with MPNs.

#### THE MPN BBOH OFFERS PATIENTS AND CAREGIVERS INFORMATION ON:

- Understanding Myeloproliferative Neoplasms (MPNs)
- Polycythemia Vera (PV)
- Primary Myelofibrosis (MF), Post PV-MF and Post ET-MF
- General Resources for Living with MPNs
- Essential Thrombocythemia (ET)



To view and download the MPN Edition of the Building Blocks of Hope go to:  
<https://www.mds-foundation.org/the-myeloid-continuum-of-diseases/>.

**EACH PATIENT HAS THEIR OWN UNIQUE STORY**

**MEET MPN PATIENT**  
**MICHELE RILEY**

## ME, GOD AND THE CLEMSON TIGERS

### MICHELE RILEY

*Brentwood, Tennessee*

Simply put, my cancer story is about having faith and trusting your instincts. Of course there are many episodes and a wonderful and important supporting cast and crew. I am the star in this story, but I am also the director. My doctor is the director of photography. He has the "vision" of how to orchestrate my treatment plan. But all decisions are ultimately mine. My best advice to you is: Always be the director in your story. In other words, always have an active roll.

My story begins at the end of 2006. I found myself really struggling through the holiday season. I was 38 and finding it really hard to complete holiday tasks, do my usual weekly errands, and work full time as a registered nurse. At first, I told myself, "Well, you are approaching 40. I guess this is to be expected." But when I still felt really fatigued after the holidays were over and I woke up a few times with

sudden but brief chest pain in the middle of the night, I went to my doctor and said, "Something is wrong". The EKG was normal so my doctor sent off some blood work. The next morning she called to tell me that there must have been some error at the lab because the results were "weird". She wanted me to come back for a redraw. Two days later she called and said, "Your repeat bloodwork came back even more abnormal. Your CBC is extremely elevated and that means there is a hematological event happening. We need to get you in to see a specialist ASAP". Her voice faded and everything around me blurred into the background. My Cancer Story had begun.

My official diagnosis came in March 2007 after a slew of tests. I remember the lab tech making slides with my bone marrow and wanting to grab them up and hide them. I knew people were going to look at those slides under a microscope and pick them apart and list all the irregularities and that made me sad. They determined I had Myelofibrosis (MF), the most serious of the Myeloproliferative Neoplasms (MPNs). No definitive cure or real treatment at the time. The good news was that MF is usually very

slow progressing. The bad news was that there was nothing they could do about the fatigue. It was now my new normal. My doctor, Dr. C, told me, "Only God knows your expiration date. So go out there and live your life fully!" He told me there was promising new research happening which would hopefully mean treatment options in 5–7 years. And stem cell transplant would be our back-up plan. He thought I could coast for 5–10 years. I decided I would look at my journey with optimism. My parents gave me a T-shirt that said "Attitude is EVERYTHING! Pick a good one!". Instead of a dramatic single movie story with a sad ending, I was going for a sitcom with many seasons.



## MPN PATIENT STORY

In Season 1, I focused on learning new ways to stay as healthy as possible and finding better ways to balance my life. The season included a 2-parter where through a crazy turn of events, I found out Dr. C and I both belonged to the same meditation group. It always warmed my heart on the nights when I looked up from my cushion to find Dr. C on the cushion beside me. It was a hug from God. I felt confident in my team and treatment plan and periodically God sent signs to reassure me. By Season 2, I had lost perspective of how fatigued I actually was. I just went on living a crazy but very happy life.

I made it to Season 6 with very little change in my health. Then my hematocrit became really high and my very thick blood caused me to be very fatigued. I began going once a month to have 500 ml of blood removed. I'd have to receive a fluid bolus to replace the volume or I'd pass out. I continued to feel weak, and the resulting low iron from the frequent phlebotomies caused constant irritable leg syndrome. I never slept well. Life was difficult.

Over time, my symptom load gradually increased and in Fall 2014 it reached a life altering level. Dr. C had retired and in a magical episode in Season 8, God directed me to Dr. S. In 2015, Dr. S started me on the drug Jakafi. It was one of the discoveries resulting from that research Dr. C had told me about in 2007. Season 8



was like a new beginning. Living a low symptom life was AMAZING!! I was able to volunteer as a mission nurse in Honduras again, and run in 5K races. I did more traveling too. The great response to Jakafi lasted through Season 10. And then the symptoms started drifting back and my blood started showing new changes. We were at a crossroads.

Before I can tell you about Season 11, I need to do a little recap. In summer 2016, when I still was in that low symptom phase, I was at work. My coworkers were discussing the upcoming season of college football. John was being boastful about his Louisville Cardinals and Jenn was praising her beloved Clemson Tigers. I never had followed football of any kind, so I was not participating in their conversation.

Eventually John insisted I join in and at least pick a side if I didn't have my own team to support. Simply to antagonize John, I said, "Okay, I choose the Clemson Tigers". I didn't even know what part of the country Clemson was in! I did a little research about Clemson, SC, the University, Coach Dabo Swinney and the players. I started getting attached to those Tigers. I also studied the rules of football. My intention was to learn enough so I could adequately razz John. But Clemson was doing well that season and I quickly got drawn in. I told him "We" (Clemson) had "Tiger Power" and we would win the National Championship. The crazy thing is, WE did!! From then on I was hooked. I used the phrase "Tiger Power" to proclaim the reason behind victories for Clemson or myself personally. #TigerPower became part of my identity.

Now back to spring 2017. Things were changing and not in a good way. Dr. S gave me options. My gut said "No" on SCT. He had a brand new clinical trial that he was especially excited about but he was hesitant about enrolling me. The new trial involved paring the Jakafi I had been taking with an investigational drug. These drugs had never been paired before, and Dr. S said there was a risk it could cause harm. A low risk, but still a risk. He did have a patient in the trial that had recently achieved remission, so it was worth at least thinking about. I went home and started



researching the investigational drug. It was not yet named so it was known by the alphanumeric the FDA had assigned to it. I didn't notice it right away, but in the name was a sign. When I saw it, I knew instantly that I had to enter that trial. The investigational drug was TGR-1202. TGR as in Tiger! It had Tiger Power!! God had set in place the series of events that led to my becoming a Clemson Tiger fan so I'd see the "Tiger Power" sign and not be afraid of the clinical trial. It all made sense that I had suddenly become a college football fan at the age of 47! I told Dr. S my Tiger Story and that 2 was my favorite number. "I will be Patient #2 in remission!" "You just watch", I told him. He chuckled and said, "Okay, let's go for it!" I started the clinical trial in July 2017 and wore my Clemson Tigers T-shirts and sometimes tiger ears to my weekly clinic appointments. Dr. S calls me Smiley Riley, and soon the staff all knew about Smiley Riley in the tiger ears who insisted she would achieve remission. Everyone was so supportive! I have photos of some of them in Tigers ears too! By November, to everyone's delight, I had, in fact, reached complete remission!! I've enjoyed telling my Tiger Story over the years. If you keep an open mind, I believe

there are messages for all of us to help guide us on our paths.

Complete remission means complete cessation of symptoms. My CBC is now normal and my precious bone marrow has no irregularities for someone to list. I feel better today than I did in my thirties. I remain in the clinical trial for now and recently passed the remission 2 year mark. We are not sure what the future holds. We will keep riding this wave for now. Season 12, 13 and now 14 is all about living a full life without limits. I joined Iron Tribe Fitness and do High Intensity Interval Training (HIIT) under the careful watch of my coaches. I run, jump up on boxes, lift weights and do real pushups! I also trained for and ran a half marathon. I celebrated my 50th Birthday in Ireland with friends. An important Bucket List achievement. I became a Clemson donor in 2018. College Football is now one of my passions. Me, Jenn and John have started the tradition of going to the Clemson vs. Louisville game together with our families each season. Life is good and full of new opportunities!

My doctor and I decided that we needed a patient support group in our part of the country. He wanted me to include



MDS along with MPNs because they have recently discovered that these diseases overlap. They are like cousin diseases. So I started a private Facebook page called Nashville MPN/MDS Support Group for patients in Tennessee, Southern Kentucky, Northern Mississippi, Northern Alabama and Northern Georgia. Right now we offer support via the group page only but we hope to hold meetings several times a year once we have enough interest in formal meetings. I also plan to try Facebook live sessions in 2020 as an additional way to connect patients. I sit on the MPN Research Foundation Impact Council to help the MPNRF identify patient needs and provide feedback regarding projects and outreach activities. I recently attended an MDS Foundation Patient and Family Forum in Nashville to educate myself about MDS. I am hoping to find a few MDS patients in my area to help me with the group and to be administrators on the Facebook page. I have received so much support from so many wonderful people over the years. I would like to pay it forward and help as many patients as I can.

**One of the mottos at Iron Tribe Fitness is "Stronger Together". I think that applies for Cancer patients too.**



## MDS/MPN-UNCLASSIFIED AND THE SCIENTIST

**JOHN SOPER, PhD**  
Yukon, Oklahoma

While I've been generally healthy for most of my life, I've had several instances of elective or emergency hospitalizations. The first of these involved a bleeding ulcer in which my hemoglobin dropped to around 6-7 g/dl. I was treated in a hospital in California, at the very beginning of the AID's crisis. Although there were no tests for AIDs, I was going into shock and received my first transfusion.

Several years later, I had another ulcer, and received my second transfusion. After this I began being much more conscientious about routine physicals, and became a 4 gallon Red Cross blood and platelet donor. I was also continuing to battle lifelong weight, cholesterol and high blood pressure issues.

In January of 2014, I discovered that I had atrial fibrillation. After running many tests, my cardiologist performed a cardioversion to correct the issue. While this seemed to be successful, a few weeks later I began experiencing some angina like symptoms. After performing an angiogram, we realized that I needed a quintuple coronary bypass.

A few months after surgery, my cardiologist told my primary care doctor, that I had macrocytic anemia. My primary care doctor realized that I needed to see a hematologist.

Dr. Abby Bova had already taken care of my wife's breast cancer, and we knew that she was the perfect fit for us. She quickly ordered every conceivable test for me, including a bone marrow biopsy.

When my wife and I finally saw Dr. Bova she was literally beaming with joy! She told us that all of the tests were negative.

Unfortunately, I still had macrocytic anemia. I would see her every three months, and all test results, except for those concerned



*Christine and John Soper*

with the anemia, seemed to be consistently in the "Normal-Low Normal" range.

When we returned to Oklahoma City, Dr. Bova repeated all of her earlier lab tests, including a new bone marrow biopsy. This time the results showed that I had 4 mutations, as well as altered chromosomal results. My LDH levels were substantially elevated, my neutrophil count continued to rise, and we were now detecting blasts in my peripheral blood.

Dr. Bova wanted to prescribe "Jakafi" (Ruxolitinib), as well as "Vidaza" (Azacitidine). She found that there was



*John with his youngest daughter, Dannie*

only one Phase 1 trial in the country using them. The trial was at MD Anderson in Houston. She contacted Dr. Naval Daver, lead investigator in the trial, and submitted all of the data she had on my case.

MD Anderson contacted me in just a couple of days. We drove to Houston, repeated all of my original testing and confirmed all original tests. I returned to Houston in two weeks and was now officially admitted to the study. I had an official diagnosis of MDS/MPN-unclassified.

I have now had three months of Jakafi alone, followed by another bone marrow biopsy. After completing that regimen I had three months of combined Jakafi/Vidaza therapy, followed by another bone marrow biopsy. That biopsy showed no increase in blasts, and no evidence of a clonal neoplasm. In another month I will have a third "staging bone marrow biopsy".

While my white counts are behaving nicely, I am becoming transfusion dependent. We have mutually agreed that a hemoglobin level of less than 8.0 mg/dL warrants a transfusion. I have had about 8-10 transfusions at this time. My ferritin concentration is approximately 1,500 ng/ml. We are aware that Vidaza is causing myelosuppression at this time, but we are also confident that it will eventually allow my hemoglobin levels to rise.

During my last visit with Dr. Daver, I was asking him a question about some of my genetic results. He simply said, "You're a scientist, you're aware of the statistical variability in any laboratory measurements." It is very important to me that both of my Doctors value my input, and that I am involved in making decisions that all of us are comfortable with.

I've had the opportunity to be actively involved with research at places like Johns Hopkins, Mt. Sinai, University of Arkansas for Medical Sciences, and the University of Maryland. I can't conceive of a better team, or overall medical care that I am now getting.

Let me share some final quick comments with you.

(1). As I stated earlier, there are some particular things that I intend to do. I have a very complicated initial diagnosis. As far as Dr. Bova is aware, I am the only person in the entire 4 state Mercy medical system to be receiving both Vidaza and Jakafi. I'm also the only crossover patient (MDS/ MPN) to be getting these medications. My clinical trial lasts for five years, and hopefully I may produce some interesting results by then.

(2) There are also things that each of you can do. First and foremost, your "primary caregiver", wife, husband, other family members, etc. are among your most important assets. Always try to give them as much consideration as they give you. Aggressively maintain the other valuable contacts that each of you already have. I'm convinced that the level of achievement that I was able to access in my early career came from the fact that I've been able to maintain those contacts for many years.



*Sculpture of Captain Love, an elite navigator for the Army Air Corps in World War II. When we see him we know to turn to the right to go to the hospital. I am posing in my Eagle Scout "Navy" flight jacket.*

**I REALIZE THAT NONE  
OF US KNOW HOW OUR  
MEDICAL CONDITIONS  
MAY PROGRESS,  
BUT WE CAN CHOOSE  
TO NOT LET THAT FACT  
CONTROL OUR LIVES.**

In many of your comments, and in questions we're asked during our treatments, we're reminded that spiritual support is vital to our well-being. Most of the places where I've worked, or been a patient, offer not only superior medical care, but have direct Christian or Jewish institutional support. My very close friend, and Laboratory Manager, Dr. Phil Kemp, leads a Bible Study at the Federal Aviation Administration. This is one of the most incredible groups that I've ever been involved with. The Naval Academy Chapel was also a strong reminder of the importance of a "Spiritual Anchor" to our lives.

Every day that I was a Hopkins fellow I would pass through the old main lobby, and see a 9-foot statue of "Christ the Healer". While Hopkins represents excellence in medicine, this was a constant reminder that there is a power beyond even what the world's best physicians can do.

(3) Another very important support that you have comes from your fellow patients. My original "MDS buddy" (the same one my father treated many years before), told me when we first met "I have this tattooed on my forehead, the drugs work until they don't".

Several weeks ago, two of us were getting Vidaza treatments. I asked the fellow next to me if he had MDS. He didn't know what that was. I asked him why he was receiving injections, and he told me that he had AML. When I asked him how

long he had been treated, he told me that he'd been receiving treatments for five years. I was really stunned to find that there was a fellow right next to me that had the very condition that I had thought was supposed to be "the end of the road" for MDS patients. He seemed to be doing very well to me!

## CLOSING THOUGHTS

When I think of the comments of my "MDS buddies" I realize that none of us know how our medical conditions may progress, but we can choose to not let that fact control our lives.

For my part, I am preparing to retire, and try to carry out some of the things above that I've already said that I can do. Frankly, the single most important thing that each of us can do now is rejoice in each new day that we're given and "Make Memories".

Thanks for reading "my story"; I hope you enjoyed reading it as much as I've enjoyed writing it!



*Christ the Healer at Hopkins*

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**Honor or memorialize your loved one at: [www.mds.foundation.org/donate](http://www.mds.foundation.org/donate)  
or contact us at 800-MDS-0839 (within US), 609-298-1035 (Outside US).**

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# Did You Know?

**The Myelodysplastic Syndromes (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 set up, no formal working group had been devoted to MDS. Since its inception, we have conducted 15 international symposia in Austria, England, the United States (Chicago, Washington, DC), Spain (Barcelona, Valencia), Czech Republic, Sweden, France, Japan, Italy, Greece, Scotland, Germany, and Denmark. The 16th International Symposium will be held in Toronto, Canada on May 5-8, 2021.

A major **MDS Foundation** effort is our international information network. This network provides patients with referrals to Centers of Excellence, contact names for available clinical trials, sharing of new research and treatment options between physicians, and extension of educational support to physicians, nurses, pharmacists and patients.

In response to the needs expressed by patients, families, and healthcare professionals, we have established patient advocacy groups, research funding, and professional educational initiatives.

The MDS Foundation is a publicly supported organization, exempt from federal income tax under section 501(C)(3) of the IRS code.

**Learn more about**

**The Myelodysplastic Syndromes Foundation, Inc.**

**and find additional resources here:**

**[www.mds-foundation.org](http://www.mds-foundation.org)**



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**Leah**

Celgene Patient Support® Specialist

# ARE BLOOD TRANSFUSIONS HOLDING YOU BACK?

If you've been diagnosed with myelodysplastic syndromes (MDS) with anemia, the phase 3 COMMANDS Trial is a clinical research study investigating a potential treatment option that may help reduce the number of blood cell transfusions you need. To learn more, talk to your doctor and visit [ClinicalTrialCOMMANDS.com](http://ClinicalTrialCOMMANDS.com).



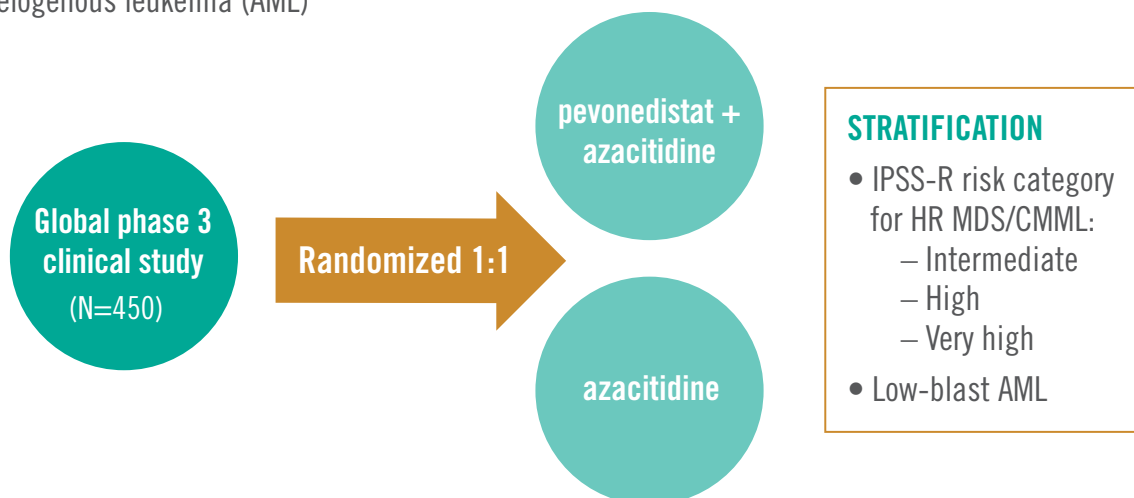
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# PANTHER TRIAL

**PEVONEDISTAT-3001** (NCT 03268954): Designed to evaluate the safety and efficacy of pevonedistat plus azacitidine versus single-agent azacitidine as a first-line treatment for patients with higher-risk myelodysplastic syndromes (HR MDS), chronic myelomonocytic leukemia (CMML), or low-blast acute myelogenous leukemia (AML)



**Primary endpoint:** Event-free survival (EFS)

**Key secondary endpoint:** Overall survival (OS)

Not a complete list of endpoints.

## Enrolling countries

Australia, Belgium, Brazil, Canada, Czech Republic, France, Germany, Greece, Israel, Italy, Japan, Mexico, Poland, Russian Federation, South Korea, Spain, Turkey, UK, USA

## For more information, including all inclusion and exclusion criteria

1-844-ONC-TKDA (1-844-662-8532) (US callers)

+1-510-740-1273 (ex-US callers)

[www.clinicaltrials.gov](http://www.clinicaltrials.gov) or [www.takedaoncology.com](http://www.takedaoncology.com)

For information on enrolling a patient, please contact [GlobalOncologyMedInfo@takeda.com](mailto:GlobalOncologyMedInfo@takeda.com).

Pevonedistat is an investigational drug. Efficacy and safety have not been established.



ONCOLOGY



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**ADVANCING RESEARCH & PATIENT CARE**

**THE 16TH INTERNATIONAL CONGRESS ON  
MYELODYSPLASTIC  
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**2021**  
**5 - 8 MAY**

**TORONTO, CANADA**



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