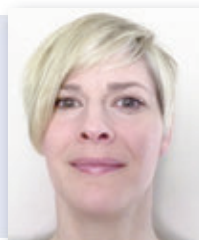


MDS NEWS HIGHLIGHTS

FROM THE GUEST EDITOR'S DESK

- **DNA Microarrays as Adjuncts to Cytogenetics:
Next Generation of Karyotyping for MDS?**
Presented by: *Tracy Tucker, PhD, FCCMG*
and *Aly Karsan, MD, FRCPC*



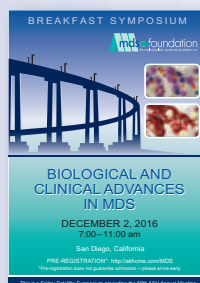
PLAN TO ATTEND OUR UPCOMING SYMPOSIA

ASH 2016 MDS FOUNDATION BREAKFAST SYMPOSIUM

December 2, 2016
San Diego, California

14TH INTERNATIONAL SYMPOSIUM ON MYELODYSPLASTIC SYNDROMES

May 3–6, 2017
Valencia, Spain



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DNA Microarrays as Adjuncts to Cytogenetics: Next Generation of Karyotyping for MDS?



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Myelodysplastic syndromes (MDS) are a group of clonal hematologic malignancies that can be difficult to diagnose morphologically. The identification of a genetically abnormal clone is particularly helpful in diagnosing MDS in difficult cases. Various kinds of genetic abnormalities also help stratify risk of progression of MDS to more aggressive disease, typically AML. The molecular and cytogenetic landscape has changed rapidly with an explosive growth of new genetic information underlying both constitutional and somatic disorders. Nevertheless, the implications of the identified abnormalities are lacking, particularly in cancer, and MDS is no exception. This discrepancy between the predicted outcomes of these novel genetic abnormalities and the ease with which the abnormalities can be identified has left an unsatisfactory gap for clinical laboratories in implementing new genetic technologies.

Standard Cytogenetic Analysis vs. Microarray Analysis in MDS

Bone marrow karyotyping has been the standard genetic analysis for MDS for decades. As a group, 40% of all primary MDS cases have a normal karyotype and another 10% have balanced abnormalities (i.e. translocation or inversions), commonly within the MLL and EVI1 genes. Therefore, 50% of cases have an abnormal karyotype with abnormalities involving chromosomes 5 and/or 7 accounting for over half of these.¹

Cytogenetic abnormalities are recognized as one of the key features that predict prognosis in MDS.² As the number of MDS cases in which cytogenetics were performed increased, so did the ability to further stratify each abnormality into prognostic groups. In 2012, the International working group for prognosis in MDS (IWG-PM) improved the original 1997 international prognostic scoring system (IPSS-R) and included a refinement of the prognostic impact of cytogenetics.³ The IWG-PM studied 7012 patients and recorded all cytogenetic abnormalities and considered anything that occurred in 10 or more patients as recurrent. A multivariate analysis of cytogenetics and overall survival and risk of progression to AML expanded the cytogenetic prognostic

categories from three to five and included more recurrent cytogenetic abnormalities (Table 1).^{2,3}

Despite the improvements in the cytogenetic risk stratification in the IPSS-R, there are far fewer abnormalities in the cytogenetic risk categories compared to the multitude of those observed. By default, all unassigned abnormalities are classified as intermediate, many of which likely warrant a different classification but are not sufficiently recurrent to be classified correctly. Within the cytogenetic risk categories there is a wide range in patient outcomes. Some of the variability in outcomes among the cytogenetic prognostic categories is likely due to genetic abnormalities below the ~10 Mb resolution of a standard karyotype as well as sequence mutations. DNA microarrays offer the ability to perform a whole genome analysis for copy number abnormalities (CNAs) that can be identified by karyotype as well as many that are smaller and cannot be identified by standard karyotype analysis. The use of DNA microarrays in MDS has shown numerous cryptic CNAs, many of which occur within genes known to have significant impact on prognosis.⁴⁻⁷ However, microarrays would not be able to identify cases with balanced translocations and using this analysis alone would miss prognostically significant abnormalities.

Table 1. IPSS-R Cytogenetic Prognostic Scoring System

Prognostic Subgroups	Cytogenetic Abnormalities
Very Good	-Y, del(11q)
Good	Normal, del(5q), del(12p), del(20q), double abnormalities including del(5q)
Intermediate	del(7q), +8, +19, i(17q), and other single or double independent clones
Poor	-7, inv(3)/t(3q)/del(3q), double abnormalities involving -7/del(7q), up to 3 karyotype abnormalities
Very Poor	Greater than 3 karyotype abnormalities

It is not surprising that microarrays can identify abnormalities below the resolution of a standard karyotype, but some studies comparing microarrays and karyotype analysis have shown that microarrays identified abnormalities that should typically be identified by a karyotype and were missed.^{7,8} This discrepancy could be due, in part, to the artifact that culturing introduces. It is well known that long-term culture (>48 hours) of malignant samples will result in the outgrowth of the normal cell line over the malignant cell line. However, it is not clear how different clones within a specimen, even in short-term cultures (24–48 hours), are selected or not selected in the culture environment. It is assumed that the proportion of each cell line identified by a randomly analyzed sample reflects the proportion of the clonal population in the sample as a whole. However, the artificial environment provided by the culture medium may not provide equal opportunity for all cells to proliferate, and some clones may not be assayed at all. Thus the advantage of single cell analysis provided by karyotyping also leads to potentially missing clones that do not survive and divide in culture.

Microarrays have the advantage of not requiring cell culture. A microarray has the potential to identify genetic abnormalities in different clones regardless of their ability to proliferate in culture. The disadvantage is that the relationship of these abnormalities to each other is lost with microarrays. Genetic clonal evolution is often regarded as evidence for disease progression and therefore is associated with a poor prognosis. In contrast to standard karyotyping, microarray analysis uses DNA from the bulk population, therefore masking the potential to determine whether subclones have genetically evolved. However, methods have been developed to estimate clonal evolution by inferring the proportion of each of the different cell lines from the differential representation seen in the microarray readout, or from the addition of SNP probes.⁹ Although promising, this type of analysis requires further validation.

Microarrays have the ability to identify abnormalities that could impact prognosis and/or treatment in a specimen that would have failed cytogenetic analysis.

The limitation of microarrays to identify subclones is related to the higher level of mosaicism relative to a karyotype that is necessary to be reliably detected with a microarray. At diagnosis, 20 metaphases are karyotyped,¹⁰ and this is assumed to exclude a level of mosaicism as low as 14% with 95% confidence (Hook 1977). Generally speaking, 20% mosaicism is the lowest level microarrays can detect, although several studies suggest this could be much lower if SNPs are included on the microarray. If a lower threshold can be achieved with advances in chemistry, probe design or analysis software, the level of mosaicism that could be excluded with microarrays might approach that of karyotype analysis.

One of the simplest benefits of microarrays over karyotype analysis is the ability to identify genetic abnormalities in specimens that have not produced dividing cells that are necessary for karyotype analysis. From a laboratory standards perspective, up to 5–10% of bone marrow specimens can fail to produce dividing cells for analysis or sufficient material for analysis.¹¹ There is a high failure rate among fibrotic marrows where the material is limited. Microarrays have successfully been performed on samples that otherwise would have failed¹² and shown the benefit of a DNA based analysis on a limited specimen over karyotype analysis. Thus, microarrays have the ability to identify abnormalities that could impact prognosis and/or treatment in a specimen that would have failed cytogenetic analysis.

The Future of Genetic Analysis in MDS

The increased resolution of microarrays relative to karyotype analysis has made arrays the first line test over karyotype for developmental delay.¹³ Several studies have shown microarrays to have a similar detection rate as karyotype analysis. A multicenter, multi-microarray platform study on different hematological disorders found good correlation (76%) between the microarrays and standard karyotype and/or FISH analysis. In this study, microarray performed on MDS cases missed two unbalanced abnormalities seen on standard karyotype and the microarray identified three CNAs and one loss of heterozygosity (LOH) region not seen on karyotype. In addition, there was a good correlation of results between platforms, thereby allowing a clinical laboratory to choose a platform best suited to its workflow.⁸

The greatest benefit of microarray analysis is evident in MDS cases with a normal karyotype. Microarray analyses have identified cryptic genetic abnormalities in 10–20%^{6,7} of normal karyotype MDS cases. These cryptic abnormalities likely partially explain why some patients who have normal karyotype have poorer outcomes compared to others. Although some of these cryptic abnormalities occur in genes known to affect prognosis,^{6–8} there are far more CNA identified where the prognostic impact is unknown. However, with time and more data, the significance of this data will be resolved, similar to where karyotypic abnormalities were many years ago.

The increased resolution of microarrays and the ability to identify more CNAs, many of which have unknown prognostic significance, will result in an exponential increase in the number of patients in the intermediate prognostic group and initially will likely show poor correlation with prognosis. Rather than concentrating on individual abnormal loci identified on microarray, several studies have shown a correlation with the amount of genetic material that is abnormal by microarray and prognosis. Some have shown that the proportion of the overall genetic

abnormalities correlate with survival or treatment response.^{6,14} This correlation may represent a surrogate for genomic instability and may be an independent predictor that is not necessarily region specific. One study showed that the amount of genetic material lost correlated with treatment response.¹⁵ In another study, additional abnormalities identified on microarray analysis, that were not seen on karyotype, predicted poorer survival than when no additional abnormalities were detected.¹⁶ Further studies in MDS are necessary to define the size restrictions used to define a CNA and how the proportion of disrupted genetic material is calculated.

In cancer, the DNA for microarrays is extracted from both the normal and abnormal cells. It is clear that low level mosaicism will be a limiting factor of microarray analysis. Enrichment strategies that isolate purified cells most likely to carry the genetic abnormality will likely improve the detection rate of microarrays, as will analysis software to detect lower level clones. A purified sample would improve the detection of tetraploid cells, which is evident at clonal evolution, as these genetically abnormal cells in a diploid background create difficulty in interpreting the relative fluorescence intensity detected by microarray.⁵ Although SNP arrays can improve the detection of tetraploid cells because of the added information the allele tracks provide.

MDS is a heterogeneous disorder, as are the genetic abnormalities identified in the disorder. The number of laboratories offering sequence panel tests for genes mutated in MDS and exon level resolution microarrays is increasing. The number of target genes on these panels is increasing beyond the handful of genes that have associated treatment guidelines in MDS.¹⁷ It is clear that one type of analysis will not identify all prognostically significant abnormalities. Microarray offers a higher resolution, whole genome analysis and can be performed with a higher throughput than conventional cytogenetics. Although microarrays cannot identify balanced rearrangements, those recurrent abnormalities with prognostic significance

could be identified with a FISH panel or RNA sequencing panel. Next-generation sequencing panels are also necessary to identify the growing list of recurrent sequence abnormalities that are not detected by microarrays and are important for assigning prognosis. While methods to detect CNA using next-generation sequencing panels is improving, microarrays offer the ability to identify larger abnormalities not conducive to detection by sequencing and too small to be detected by karyotype.

Not unlike the collaborative efforts necessary for defining cytogenetic prognostic scoring, future collaborative studies assembling the vast amount of genetic information on CNAs, sequencing and recurrent abnormalities will be necessary to determine the clinical significance and define a new prognostic scoring system. Further, complementary guidelines on how to analyse the different systems used in the analysis should be created. In particular, defining the size of the abnormalities identified by microarrays that should be reported, and which genes require a lower resolution assessment is necessary. If the amount of total genetic aberration continues to prove significant, guidelines on how to calculate this should also be included. In particular, guidelines on how the total genomic content of a whole chromosome gain or loss should be calculated relative to the same amount of abnormal genetic material in a case with multiple smaller abnormalities (e.g. chromothripsis) which is likely to be more unstable and carry a higher risk for disease progression. In addition, if SNP arrays are used for the analysis, it will be necessary to determine how loss of heterozygosity, which is identified in up to 20% of MDS cases¹⁶, should be considered and what sizes or regions of the genome should be reported clinically.

There is no doubt there will be growing pains associated with the increase in genetic information. However, microarrays offer far more information than karyotype analysis and efforts to enrich the population studied and analysis algorithms allowing lower mosaicism will improve the

detection rate of microarrays. Microarrays will not detect every abnormality, but the added benefits that microarray analysis offers over karyotype outweighs the few cases that an abnormality will be missed. The information gained from identifying new recurrent abnormalities offers the potential for better stratification of patients and potentially newer treatment options. The IWG-PM is in an excellent position to collect these sorts of data with enough statistical power to answer the question as to whether a combination of DNA microarray analysis and next-generation panel sequencing provides better prognostic stratification, which would potentially make standard karyotype analysis redundant in the future.

Glossary

Chromothripsis: A single event that results in hundreds to thousands of clustered chromosomal rearrangements in one or a few chromosomes.

Copy number abnormality: Variation identified in individuals in the number of copies of a particular DNA sequence in their genome.

Diploid: Two copies of each chromosome.

FISH: Fluorescence in situ hybridization is a technique that uses fluorescently labeled DNA probes to identify copy number changes or genomic rearrangements.

Karyotype: Analysis of the number and structure of chromosomes.

Homozygosity: Two identical DNA sequences at a particular location in the genome.

Heterozygosity: Two different DNA sequences at a particular location in the genome.

Loss of heterozygosity: Homozygosity in a tumour cell when constitutionally the individual was heterozygous at the same location.

Microarray: DNA sequences (probe) are attached to a solid surface and are used to hybridize a fluorescently labeled patient DNA sample (target) under high-stringency conditions. The fluorescent intensity of the probe-target hybridization is quantified and used to determine relative abundance of a DNA sequence in the patient.

Mosaicism: Two or more genetically different cell lines. These differences could be sequence mutations or copy number changes.

RNA: Ribonucleic acid that is generally single stranded and plays a role in transferring information from DNA to protein.

SNP: Single nucleotide polymorphism is a position in the genome where two or three alternative nucleotides are common in the population.

Tetraploid: Having four copies of each chromosome.

Translocation: Transfer of chromosomal regions between two different chromosomes.

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UPCOMING MEETINGS

THE AMERICAN SOCIETY OF HEMATOLOGY 58TH ANNUAL MEETING & EXPOSITION • DECEMBER 2016

On behalf of the MDS Foundation and our Board of Directors, please join us for our upcoming Satellite Symposium:

SAVE THE DATE

Biological and Clinical Advances in MDS

Friday, December 2, 2016, 7:00–11:00 am

San Diego, California

This is a Friday Satellite Symposium preceding the 58th ASH Annual Meeting.

VISIT THE
MDS BOOTH:
3027



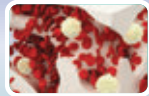
TARGET AUDIENCE

This activity is intended for physicians, oncology nurses, nurse practitioners, physician assistants, pharmacists and other health care professionals interested in the treatment and management of patients with Myelodysplastic Syndromes.

LEARNING OBJECTIVES

- Describe the new WHO classification of MDS
- Describe molecular features which are useful for classifying MDS and aiding in diagnosis and therapeutic decision-making
- Describe differences in therapy-related MDS which need consideration for their treatment
- Describe immune-related mechanisms and treatment approaches for patients with MDS
- Describe novel biologic approaches for treating anemias associated with MDS

BREAKFAST SYMPOSIUM

**BIOLOGICAL AND
CLINICAL ADVANCES
IN MDS**

DECEMBER 2, 2016
7:00–11:00 am
San Diego, California

PRE-REGISTRATION*: <http://akhcme.com/MDS>
*Pre-registration does not guarantee admission—please arrive early.

This is a Friday Satellite Symposium preceding the 58th ASH Annual Meeting.



THE 14TH INTERNATIONAL
SYMPOSIUM ON
**MYELODYSPLASTIC
SYNDROMES**

2017

MAY 3 - 6
Valencia, Spain

**ADVANCING
RESEARCH &
PATIENT CARE**

Welcome to MDS 2017



Guillermo Sanz

On behalf of the Scientific and Local Organizing Committees and the MDS Foundation, it is our pleasure to invite you to the 14th International Symposium on Myelodysplastic Syndromes taking place in Valencia, Spain from May 3-6, 2017. As in previous years, the Symposium will cover all relevant clinical aspects of MDS diagnosis, prognosis, and management as well as the newest data in MDS basic and translational research. The main lectures will be delivered by recognized international leaders but we also expect to include high-level research talks selected from the abstracts submitted by attendees. Further, we are very happy to offer you the opportunity to visit Valencia, a city with a perfect combination of tradition and modernity. Located in the East of Spain, on the shores of the Mediterranean Sea, Valencia has a unique charm and is one of the cities in Europe that has experienced the most significant growth over recent years in terms of events and international recognition. This is due to the renovation of the historical city center and the creation of new cultural and environmental sites such as the City of Arts & Sciences and the Oceanographic Marine Park. Valencia has mild weather all year long with more than 300 days of sunshine per year, more than 20 km of beaches, a varied gastronomy and the home of the paella, and a Mediterranean way of life. The venue, the Valencia Conference Center, is a modern building designed by Norman Foster and is ideally located. It takes only 10 minutes to reach the historic city center (excellent links by metro, bus and tram), is only 5 km (10 minutes) from the international airport at Manises, and offers over 1,000 hotel rooms within walking distance.

We look forward to seeing you in Valencia!

Guillermo Sanz
Symposium Chair



MDS 2017 Symposium Secretariat:
c/o Kenes International
Email: mds@kenes.com
For MDS Foundation Contact:
US number: 1-800-MDS-0839
Outside the US: 1-609-298-1035

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ADVANCING RESEARCH & PATIENT CARE

2017

MAY 3 - 6

Valencia, Spain

MDS 2017 will attract an international audience of researchers, clinicians, scientists and educators from around the world who deal with MDS.

The MDS 2017 Symposium includes presentations delivered by renowned professionals on the latest developments in hematology.

MDS 2017

THE 14TH INTERNATIONAL SYMPOSIUM ON MYELODYSPLASTIC SYNDROMES



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INTERNATIONAL WORKING GROUPS

MAKING CONNECTIONS AROUND THE WORLD

The MDS/MPN International Working Group

Myelodysplastic syndromes represent a heterogeneous group of disease entities with diverse clinical features, genetic composition, natural history, and response to therapy. Mounting evidence has suggested that several MDS ‘subtypes’ are distinct enough that they should be considered unique disease entities. To this end, in 2008 the World Health Organization designated four clinical entities to be recognized as bona fide diseases with overlapping dysplastic and proliferative features. These include Chronic Myelomonocytic Leukemia (CMML), atypical CML (aCML), Juvenile Myelomonocytic Leukemia (JMML), and Myelodysplastic/Myeloproliferative Neoplasms Unclassifiable (MDS/MPN-U). Since this reclassification, many investigations have confirmed the unique molecular underpinnings and clinical trajectories of each of these diseases. However, this stratification has resulted in rare diseases that require collaborative efforts to make transformative changes in patient care.

The MDS/MPN International Working Group (MDS/MPN IWG) was originally developed in 2012. The work of this initial group resulted in the first two peer-

reviewed publications. By the end of 2013, membership was expanded to include a CMML multi center project, and the group enlisted the support of the MDS Foundation. The overarching goal of this group is to identify key knowledge gaps in the area of MDS/MPNs and facilitate international, collaborative, translational science geared to rapidly improve our understanding of these fatal neoplasms. The current membership includes 32 investigators, from 20 centers, across 7 countries.

Work from collaborations within this group has resulted in several peer-reviewed publications:

- A consensus recommendation for response criteria that sets the foundation for a common endpoint across many MDS/MPN clinical trials.²
- A consensus review on the biology and clinical presentation of MDS/MPNs.²
- The development of an international CMML dataset that includes clinical and molecular data.³

Ongoing collaborations underway include:

- Expansion and prospective molecular sequencing of the international CMML data set.
- Exploring the consequence of an MDS/MPN diagnosis on quality of life.



- Identify/Generating a consensus CMML prognostic model.
- Exploring the role of transplant in molecularly defined CMML subtypes.
- Implementing international clinical trials on both sides of the Atlantic.

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MDS FOUNDATION INTERNATIONAL WORKING GROUP FOR PROGNOSIS IN MDS

Latest News Regarding the Molecular Mutation Project of the IWG-PM

Mutations Predict Prognosis Independent of the IPSS-R: Overview

The International Prognostic Scoring System (IPSS) and IPSS-R were developed by the International Working Group for Prognosis in MDS (IWG-PM) under the aegis of the MDS Foundation and have become the dominant clinical tools for



predicting prognosis in patients with myelodysplastic syndromes (MDS).¹ A prognostic scoring system that integrates gene mutations into the known critical clinical features would have great additive utility for improved determination of prognosis in patients with MDS and has the potential for widespread clinical use. The ongoing project of the IWG-PM Molecular Committee (IWG-PM-M) has shown, with

the IPSS-R and other scoring systems, using larger molecularly characterized datasets, that mutations are independent predictors of patients' overall survival. This finding justifies a prognostic scoring system that will integrate clinical and genetic features.

Prognostic Impact of TP53 Mutations

A central aim of the IWG-PM Molecular project is to develop a large database of MDS patients with deep clinical annotation and genetic sequencing data for clinical, biologic and possibly

therapeutic purposes. In addition to the analysis of previous samples, sequencing additional MDS cases will be performed to further develop the database.

As a first project for the IWG-PM molecular database, the impact of TP53 mutations in MDS demonstrated that this status divides MDS patients with complex karyotypes into distinct prognostic risk groups, with those carrying the mutation having poorer prognoses. Despite their strong associations with adverse clinical and cytogenetic abnormalities that are already incorporated into existing prognostic scoring systems, TP53 mutations carry significant independent prognostic value for decreased survival for patients with MDS. This work was presented by Dr. Rafael Bejar at the 2014 American Society of Hematology Meeting² with updating at the 2015 13th International MDS Foundation Symposium held in Washington, D.C.

Recent Molecular Results

Recently, molecular and clinical data on 3392 MDS patients gathered by members of the IWG-PM-Molecular Committee were combined and analysed and the abstract describing these findings was presented at for oral presentation at the recent ASH Annual Meeting in Orlando.³ Survival data were available for 3200 patients. The 27 genes sequenced in at least half of the cohort and mutated in >1.5% of samples were included for analysis. Mutations in 12 genes were strongly associated with shorter overall survival in univariate analyses. The large size of the cohort allowed for more precise estimates of survival in the less frequently mutated genes. IPSS-R risk groups could be determined for 2173 patients and were strongly associated with survival. Adjusting the hazard ratio of death for IPSS-R risk groups identified several mutated genes with independent prognostic significance. Patients without mutations in any of the major adverse genes represented over half of the fully sequenced cohort and had a longer median survival than patients with adverse mutations even after correction for



Dr. Peter Greenberg speaking at the IWG-PM meeting during the 2015 ASH Congress.

IPSS-R risk groups. A mutation score based on survival risk will be proposed and internally validated. The impact of somatic mutations in patients traditionally considered lower risk will also be explored.

Current Project Status, Plans for sequencing of new samples

In addition to the above assessment of previous samples, led by Dr Elli Papaemmanuil, the project is sequencing additional large numbers of MDS cases to further develop our database and mutational evaluations. An automated sample management system was recently implemented that links sample reception to library preparation and sequencing submission. The results of these analyses will serve as the template with which to build an integrated molecular risk model for MDS.

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

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3. Bejar R, Papaemmanuil E, Haferlach T, Garcia-Manero G, Maciejewski JP, Sekeres MA, Walter MJ, Graubert TA, Cazzola M, Malcovati L, Campbell PJ, Ogawa S, Fenaux P, Hellstrom-Lindberg, Kern W, Boulwood J, Pellagatti A, Bowen D, Tauro S, Groves M, Vyas P, Quek L, Nazha A, Thol F, Heuser M, Shih L-Y, Padron E, Sallman D, Komrojki R, List A, Santini V, Fontenay M, Campbell P, Tuechler H, Stevenson D, Neuberger D, Greenberg P, Ebert BL. Somatic Mutations in MDS Patients Are Associated with Clinical Features and Predict Prognosis Independent of the IPSS-R: Analysis of Combined Datasets from the IWG-PM-Molecular Committee, ASH 2015 abstract, Orlando, December 2015. *Blood*. 2015;126 (23), #907.



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MDS RESOURCES

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.

EPIDEMIOLOGY, DIAGNOSIS AND PROGNOSIS:

1. Buckstein R et al. Patient-related factors independently impact overall survival in patients with myelodysplastic syndromes: an MDS-CAN prospective study. *Br J Haematol*. 2016 March 15, [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26991631>)
In 445 consecutive patients with MDS or CMML within a multicenter national registry in Canada, clinical outcomes were assessed in relation to frailty, disability, and physical functioning. Overall survival was significantly shorter for patients with higher frailty and comorbidity scores, any disability, impaired grip strength and timed chair stand tests. The multivariate analysis demonstrated independent prognostic value of adding frailty (HR-2.7, $p<0.0001$) and Charlson comorbidity score (HR-1.8, $p=0.01$) to revised IPSS (IPSS-R).
2. Park E et al. Nationwide statistical analysis of myeloid malignancies in Korea: incidence and survival rate from 1999–2012. *Blood Res*. 2015;50(4): 204–217 (<http://www.ncbi.nlm.nih.gov/pubmed/26770948>)
With overall 3771 cases of myeloid diseases (1.7% of all cancers) within the Korea Central Cancer Registry, the present study compared five year survival rates between 2001–2005 vs 2008–2012 periods. While the survival rates appeared to improve with Acute Myeloid Leukemia (AML-26.3% to 34.8%), Acute Promyelocytic Leukemia (APL-51.6% to 69.6%) and myeloproliferative neoplasms

(MPN-81.8% to 87.1%). In contrast for the same time intervals survival did not change for MDS (45.6% and 44.4% respectively)

3. Wlodarski M et al. Prevalence, clinical characteristics and prognosis of GATA-2 related myelodysplastic syndromes (MDS) in children and adolescents. *Blood*. 2016;127(11):1387–1397 (<http://www.ncbi.nlm.nih.gov/pubmed/26702063>)
Germline GATA-2 mutations were investigated in 426 children and adolescents with primary MDS and in 82 cases with secondary MDS from two consecutive studies of the European Working Group of MDS in childhood occurring over a period of 15 years. The incidence of germline GATA-2 mutations in primary MDS was 7% among all cases (and 15% in advanced disease), but completely absent in children with MDS secondary to therapy or acquired aplastic anemia. When data from additional 108 children was combined, Germline GATA-2 mutations were highly prevalent in patients with monosomy 7 (37% in all ages and 72% in adolescents). The overall survival and outcomes of hematopoietic stem cell transplantation did not have impact of the mutational status. Thus, germline GATA-2 mutation may not confer poor prognosis in childhood MDS.

TREATMENT:

Growth Factors:

1. Hutzschenreuter F et al. Granulocyte and granulocyte-macrophage colony stimulating factors for newly diagnosed patients with myelodysplastic syndromes. *Cochrane Database Syst Rev*. 2016, Feb 16 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26880256>)
A meta-analysis of five randomized controlled (RCT) trials for G-CSF (N=337) and two RCTs for GM-CSF (N=149) demonstrated no benefit in overall survival, progression free survival, progression to AML or in incidence of infections and PRBC transfusions. Larger data is needed to ascertain these observations.

Hypomethylating Agents:

1. Takahashi K et al. Clinical implications of TP53 mutations in myelodysplastic syndromes treated with hypomethylating agents. *Oncotarget*. 2016 Feb 9 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26871476>)
One hundred sixty eight MDS patients treated with hypomethylating agents were screened for TP53 mutations. Although the overall response rate did not vary, the response duration and overall survival were significantly shorter in patients with TP53 mutations as compared to those with wild type gene (DOR- 5.7 mo vs 28.5 mo, $p=0.003$; OS- 9.4 mo vs 20.7 mo, $p<0.001$ respectively). Additionally, at leukemic progression post hypomethylating agent treatment, TP53 mutations persisted.
 2. Nazha A et al. Outcomes of patients with myelodysplastic syndromes who achieve stable disease after treatment with hypomethylating agents. *Leuk Res*. 2016; 41:43–47 (<http://www.ncbi.nlm.nih.gov/pubmed/26777537>)
Within a MDS clinical research consortium database, stable disease (SD) was defined as no evidence of progression and without achievement of any other response. While 55% patients demonstrated best response to Azacytidine or Decitabine (AZA/DAC) at 4–6 mo, additional 20% achieved response at a later time point. Patients with SD at 4–6 mo who eventually achieved CR also had a superior survival vs. those who never improved beyond SD (28.1 mo vs 14.4 mo, $p=0.04$).
- ##### Hematopoietic Stem Cell Transplantation:
1. Brierley C and Steensma D. Allogeneic stem cell transplantation in myelodysplastic syndromes: does pretreatment clonal burden matter? *Curr Opin Hematol*. 2016;23(2):167–174 (<http://www.ncbi.nlm.nih.gov/pubmed/26717194>)
This review surmises that patients with more than 10% marrow blasts should be considered for cytoreduction with therapies like hypomethylating agents prior to allogeneic SCT transplant whereas those with less than 10% blasts could proceed to transplant directly.

Novel Agents:

1. Borthakur G et al. Activity of the oral mitogen-activated protein kinase kinase inhibitor trametinib in Ras-mutant relapsed or refractory myeloid malignancies. *Cancer*. 2016 March 18 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26990290>)
This phase 1/2 study evaluated relapsed/refractory AML, high risk-MDS and CMML patients with or without N-RAS or K-RAS mutations. The most common treatment-related adverse events were diarrhea, rash, nausea and increased aminotransferase levels. The phase 2 recommended dose was 2 mg PO daily. Patients with N-RAS or K-RAS mutations showed response-20% in AML/high risk MDS and 27% in CMML as compared to 3% in patients without N-RAS/K-RAS mutations. These results thus validated targeted activity of trametinib in RAS mutated patients.
2. Garcia-Manero G et al. Rigosertib versus best supportive care for patients with high-risk myelodysplastic syndromes after failure of hypomethylating drugs (ONTIME): a randomized controlled phase 3 trial. *Lancet Oncol*. 2016, March 8 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26968357>)
A total of 299 RAEB1, RAEB 2, RAEB-t and CMML patients were randomized 2:1 (199 treated with Rigosertib vs. 100 on best supportive care). With a median follow up of 19.5 months, rigosertib treatment resulted in an overall survival of 8.2 months vs. 5.9 months on control arm (HR=0.87, p=0.33). The most common adverse events with rigosertib were anemia, thrombocytopenia, neutropenia, febrile neutropenia, and pneumonia. With the lack of survival benefit in this trial, the future studies will focus on individual high risk subgroups.

PATHOBIOLOGY:

1. Novakova M et al. Loss of B cells and their precursors is the most constant feature of GATA-2 deficiency in childhood myelodysplastic syndrome. *Haematologica*. 2016 March 24 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/27013649>)

In pediatric population, the present study reported a 14% incidence of GATA-2 mutations in advanced MDS (RAEB/RAEB-t and MDS related AML) as compared to 17% in refractory cytopenia of childhood and 0% in aplastic anemia. The GATA-2-deficient MDS patients showed profound B cell lymphopenia including B cell progenitors in blood and bone marrow.

2. Zhang X et al. T-cell large granular lymphocyte proliferation in myelodysplastic syndromes: clinicopathological features and prognostic significance. *Leuk Res*. 2016;43:18–23. (<http://www.ncbi.nlm.nih.gov/pubmed/26927701>)
A study comparing 35 MDS patients with T-cell large granular lymphocyte proliferation (T-LGL) with 36 patients without T-LGL demonstrated a significant difference in peripheral blood CD3+/CD157+ cell count (p<0.01). The presence of T-LGL proliferation in bone marrow was correlated with hypocellularity and erythroid hypoplasia. However, immunosuppressive treatment for T-LGL did not result in survival benefit.
3. Raimbault A et al. APG101 efficiently rescues erythropoiesis in lower risk myelodysplastic syndromes with severe impairment of hematopoiesis. *Oncotarget*. 2016, Feb 18 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26910909>)
Two thirds of the low-risk MDS patients may overexpress CD95 correlating with poor erythropoiesis and that CD95 overexpression may indicate resistance to Erythropoiesis Stimulating Agents (ESAs). APG101, a fusion protein of the extracellular domain of CD95 conjugated with Fc portion of human IgG1 increased the number of burst forming units-erythroid (BFU-E) derived from CD34+ cells in vitro.
4. Komrokji R et al. Autoimmune diseases and myelodysplastic syndromes. *Am J Hematol*. 2016, Feb 13 [Epub ahead of Print] (<http://www.ncbi.nlm.nih.gov/pubmed/26875020>)

Among 1408 MDS patients, 391 (28%) patients had autoimmune diseases with hypothyroidism in 12% of total MDS patients as the highest incidence. Other frequently seen conditions (≥5% prevalence) were idiopathic thrombocytopenic purpura, rheumatoid arthritis, and psoriasis. Autoimmune diseases were most common in RA or RCMD and those with low blood transfusion dependency. Overall survival (60 mo) was better and rates of leukemic transformation (23%) was lower in patients with autoimmune diseases vs. those without (45 mo, p=0.006; 30%, p=0.011 respectively).

5. Patnaik M and Tefferi A. Cytogenetic and molecular abnormalities in chronic myelomonocytic leukemia. *Blood Cancer J*. 2016;6:e393 (<http://www.ncbi.nlm.nih.gov/pubmed/26849014>)

This report reviewed frequent cytogenetic and genetic abnormalities observed in CMML patients and suggested that only nonsense and frameshift ASXL1 mutations might negatively impact overall survival. Based on such results, the current prognostic models including Molecular Mayo model and Groupe Francais des Myelodysplasies model have incorporated ASXL1 mutations as a prognostic factor.

6. Myelodysplastic syndrome macrophages have aberrant iron storage and heme oxygenase-1 expression. *Leuk Lymphoma*. 2016 Jan 12 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26758041>)

Marrow iron was found to be elevated in MDS marrows (n=67) as compared to the age-matched cytopenic marrows (n=62) and was found to colocalize with heme oxygenase (HO1) protein expression and H-ferritin in CD163+ macrophages within MDS marrows. Moreover, high HO1 expression was significantly associated with shorter overall survival among MDS patients independent of IPSS-R and transfusion history.

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.

1. Rivera G, Saramipoor Behbahan I and Greenberg P. Immune checkpoint pathways: perspectives on myeloid malignancies. *Leuk Lymphoma*. 2016 Feb 25 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26916355>)
2. Stahl M et al. Lost in translation? Ten years of development of histone deacetylase inhibitors in acute myeloid leukemia and myelodysplastic syndromes. *Expert Opin Investig Drugs*. 2016;25(3): 307–317. (<http://www.ncbi.nlm.nih.gov/pubmed/26807602>)
3. Niscola P, Mandelli F and Efficace F. Improving accuracy of prognosis in patients with myelodysplastic syndromes using self-reported quality of life data. Opportunities for a new research agenda in developing prognostic models. *Exp Rev Hematol*. 2016 Mar 24 [Epub ahead of print] (<http://www.ncbi.nlm.nih.gov/pubmed/26954305>)

The present editorial provides commentary on these researchers' prior study demonstrating value of adding patient self-reported fatigue to existing prognostic tools such as IPSS, IPSS-R, and WPSS with a significant benefit in overall survival.

We would like to thank Suneel Mundle, a member of the MDS Foundation, and Rhea Mundle for their assistance in monitoring these important peer-review publications on MDS.

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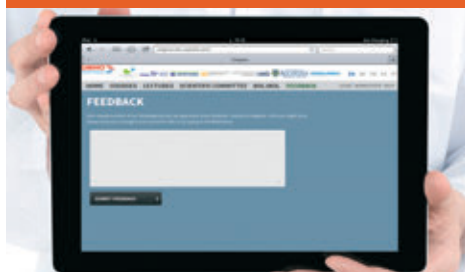
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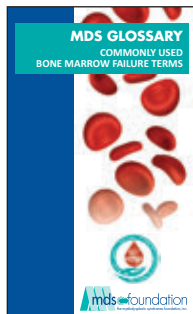
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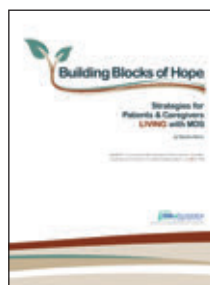
MDS Glossary – Commonly Used Bone Marrow Failure Terms

On behalf of the MDSF International Nurse Leadership Board, I am pleased to announce the creation of the *MDS*

Glossary – Commonly Used Bone Marrow Failure Terms. This glossary has been prepared to help define words, acronyms and technical terms that you may find helpful in your journey LIVING with MDS.

Building Blocks of Hope® (BBoH) – An Update

You or someone you know has been diagnosed with MDS. Hearing the words Myelodysplastic Syndrome 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. The Building Blocks of Hope® (BBoH) booklets are designed to help get you the information that you are looking for to prepare, participate, and LIVE with MDS.



The BBoH are arranged in a 6 book, easy to read format, and includes the following content:

BOOK 1 – UNDERSTANDING MDS:

A complete description of the disease process of MDS and answers to common questions.

- *What Is MDS?*
- *Is MDS Cancer?*
- *What Causes MDS?*
- *What Does Bone Marrow Do?*
- *What Are the Symptoms of MDS?*
- *The Bone Marrow Biopsy & Aspirate*
- *What Happens to Bone Marrow in MDS?*
- *Cytogenetics and Molecular Testing in MDS*

- *How is MDS Classified?*
- *How Severe Is My MDS?*
- *The Revised International Prognostic Scoring System (IPSS-R)*
- *Glossary*

BOOK 2 – SEEKING TREATMENT:

The treatment of MDS can vary based on the type of MDS you have and how severe it is. This section will provide details about the various approaches to treatment.

- *Preparing for the Initial Visit*
- *General Principles of Treatment of MDS*
- *Red Blood Cell Transfusion*
- *Platelet Transfusion*
- *Growth Factors*
- *Disease Modifying Agents*
- *What is Palliative Care?*
- *Bone Marrow Transplant*
- *The Bone Marrow Transplant Process*
- *Bone Marrow Transplant Evaluation*
- *What are Clinical Trials?*
- *Participating in a Clinical Trial*
- *Clinical Trials in MDS*
- *MDS in Children*
- *Pediatric Information Resources – MDS and Childhood Cancers*

BOOK 3 – QUICK TIPS:

The quick tips offered in this section include guidelines for monitoring and managing your symptoms.

- *Anemia in MDS*
- *Neutropenia in MDS*
- *Thrombocytopenia in MDS*
- *Fever*
- *Tracking My Blood Counts*
- *Diarrhea*
- *Constipation*
- *Nausea and Vomiting*
- *Injection Site Reactions*
- *Skin Rashes*
- *Fatigue*
- *Anxiety*
- *Depression*
- *When Should I Call My Health Care Provider?*
- *References*

BOOK 4 – IRON OVERLOAD:

Iron overload is a possible outcome of receiving repeated red blood cell transfusions. This section answers common questions, including how iron overload can be treated.

- *Blood Transfusions and MDS*
- *What is Iron Overload?*
- *How Serious is Iron Overload?*
- *Is Iron Overload Treatable?*
- *Chelating Agents Used to Treat Iron Overload*
- *What Can I Do to Avoid Iron Overload?*
- *Resources*
- *References*
- *Living With Iron Overload: Patient Testimonial*

BOOK 5 – MY MDS PLAN:

Understanding the diagnosis of MDS will help you and your caregiver take an active part in your individual treatment plan. My MDS plan provides several tools to allow you to track and manage your journey. You may want to make extra copies of some of these tools before writing on them so that you can continue to track your progress.

- *Taking an Active Part in Your Care*
- *Tracking Your Treatment*
- *My Health Profile*
- *Frequently Asked Questions*
- *Suggested References*
- *Staying Well*
- *Selected References*
- *Insurance and Reimbursement Resources*
- *Write Your Legislatures*
- *Additional Resources*
- *Living with MDS: Patient Testimonials*

BOOK 6 – THE MDS FOUNDATION:

The MDS Foundation is an international publicly supported organization dedicated to serving the MDS patient, their caregivers, and the professionals that are working to improve the lives of patients living with MDS. The MDS Foundation provides a number of resources which support the Building Block of Hope program.

- *About the MDS Foundation*
- *The MDS Foundation Staff*
- *The MDS Foundation Board of Directors*
- *International MDS Foundation Nurse Leadership Board*
- *MDS Centers of Excellence*
- *Patient Information Resources*
- *The MDS Foundation Patient Services*
- *Create Your Own Support Group*
- *Support Group – Taking it Further*
- *Planned Giving*

To view the video tutorial or to download the chapters for printing, please use the following link: <http://www.mds-foundation.org/bboh2/>.

To view and download translated editions of the handbook, please access this link <http://www.mds-foundation.org/bboh/#International-Handbooks>.

We will continue to translate and adapt the BBoH into multiple languages in order to reach as many MDS patients as possible. It is now available in Canadian English, Canadian French, Chinese, French, German, and Turkish. Current translations in process: Armenian, Australian, Danish, Dutch, Japanese and Spanish.

Allow yourself time to adjust to the diagnosis of MDS. Take time to explore the Building Blocks of Hope®. We wish you the best in your journey, and hope that this program will provide you and your caregivers with tools and strategies for **LIVING** with MDS.

And look for a new and exciting BBoH initiative coming soon to further assist patients and caregivers LIVING with MDS!!!

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MDS Foundation: Creating A Safe “Space” in Which to Educate Patients and Caregivers

Beth Fand Incollingo

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Imagine a beautiful expanse in the Southwest, a desert landscape stretching across sandswept miles until it reaches mountains that rise into the starry night sky.

The red, teal and tan logo of a sprouting plant that accompanies a comprehensive educational package aimed at patients with myelodysplastic syndromes (MDS) is designed to conjure that vision and the feelings it evokes: welcome, warmth, stability, healing, passion and protection, according to the nonprofit organization behind the effort, the MDS Foundation, Inc.

Building Blocks of Hope — Patient and Caregiver Strategies for LIVING with MDS (BBoH) is a free global print and online patient advocacy initiative that, through education, aims to foster feelings of comfort in those with the disorders and their caregivers.

The resource was developed in 2012 by Sandra Kurtin, an oncology nurse practitioner at the University of Arizona Cancer Center who is also an MDS Foundation Board member. Since then, it's been continually updated to reflect the ongoing changes in patient care and treatment.

Due to demand from patients and their families, as well as from health care practitioners, BBoH is available not only in English, but also in Canadian English, Canadian French, Chinese, French, German and Turkish. In addition, the resource is being translated into Armenian, Australian, Dutch, Japanese and Spanish, says Susan Hogan, operating director of the New Jersey-based foundation.

“It took me three days, but I have read the entire BBoH book,” one reader wrote in

a testimonial on the MDS Foundation's website. “What a magnificent undertaking on your part. The book is the most comprehensive source I have read. My understanding of MDS and possible treatment options has been greatly increased.”

Myelodysplastic syndromes, or bone marrow failure disorders, occur when bone marrow does not produce enough healthy blood cells. Too many blood cells remain immature, either accumulating in marrow or leading to a dangerous shortage of mature blood cells. The diseases can lead to low counts of red or white blood cells or platelets. In turn, this can cause side effects including infection, anemia, spontaneous bleeding or easy bruising. And for nearly one-third of the patients diagnosed with MDS, this type of disorder will progress to acute myeloid leukemia (AML).

BBoH teaches about the diseases through a variety of media: printed and digital materials; videos; educational slide sets; and links to online resources and practical tools.

The best place to start, the organization suggests, is the BBoH handbook, which provides resources, information and tools to help patients and caregivers through their journeys. The handbook explains MDS, discusses treatment options, offers tips on how to monitor symptoms and when to report them to health professionals, and provides a planning section to help patients and their caregivers track and manage their specific case information.

The MDS Foundation distributes printed copies of the BBoH on a print-as-needed basis to ensure that all copies contain the most updated information available when they are released. Online, the resource can be found at mds-foundation.org/bboh; for copies in languages other than English, visit mds-foundation.org/international-handbooks.

One advantage to accessing the handbook online is that patients can answer some questions and then get a personalized version of the book.

Besides the BBoH effort, Hogan says, the MDS Foundation conducts international symposia for researchers, clinicians,

scientists and educators; the 14th International Symposium will be held in Valencia, Spain on May 3–6, 2017. And it hosts not only patient support groups, but free one-day conferences for patients and caregivers living with MDS. Information about these events can be found at mds-foundation.org/patient-and-family-forums.

The foundation also offers an international information network, which refers patients to centers of excellence and connects them with clinical trials, and provides doctors with opportunities to discuss new research and treatment options and get educational support.

Finally, Hogan says, the foundation provides research funding in the form of two-year, \$50,000 grants to investigators 40 years of age or younger who are pursuing basic research or clinical study of the causation, epidemiology, molecular biology, cytogenetics, morphology, prognosis and/or management of MDS. Two such awards were given by the MDS Foundation in 2015, and the organization hopes to offer an additional grant in 2016.

The MDS Foundation can be reached at 800-MDS-0839 within the United States, online at mds-foundation.org and by email at patientliaison@mds-foundation.org.

When it comes to the BBoH effort, with every handbook that reaches a patient or caregiver, the MDS Foundation hopes to spread not just education and comfort, but hope. It's another one of the principles reflected in the logo for the program.

The graphic is “constructed in a wave-like pattern indicating the fluidity of life, health and illness,” organization literature explains. “The single red band which continues up into the plant symbolizes strength and improvement in bone marrow function. The idea of hope for the future and extension of life is emulated in the sprouting plant.”

See more at:

<http://www.curetoday.com/share-your-story/mds-foundation-creating-a-safe-space-in-which-to-educate-patients-and-caregivers?p=2#sthash.8cJvemfw.dpuf>

FROM THE FOUNDATION

TESTIMONIALS

"Best people on the planet. This place is wonderful and needs more recognition!"

Not everything's about the mighty dollar. Some people actually do care! GOD Bless the MDS Foundation!
Scott R.

"This is a wonderful organization."

I'm so grateful my daughter found this MDS Foundation for me.
Mary P.

"The embodiment of what a patient-support organization should be!"

Thank you Audrey Hassan for your expert and unfailing support!
Lynn M.B.

"Your first chapter of "Building Blocks of Hope" is fantastic; very informative, very reassuring!"

Beverly E.

"Wonderful organization and they were so helpful when Ken was first diagnosed."

Janet K.

"As a daily caregiver & companion of an MDS patient, I'm so glad we found you."

Your organization has been a great support system for both of us as we knew so little. I pray for all of you and hope someday there will be a cure. As for now, my friend is faced with the decision of a full year of chemo that will not be a cure. I still don't understand why age is such a factor regarding bone marrow transplants.

Luann B.

"When my brother was diagnosed in 2004, we had no idea what this awful disease was."

He passed away two years later, and I have been learning about it thanks to your site. God Bless you!"

Jaki T.

"Great Foundation that is not all about raising money."

Bob M.

"Thank you!"

Blessed be to your foundation on giving people the proper knowledge of the medical condition. Blessed be everyone at the MDS Foundation.

Oscar M.G.

"I have problems keeping up with MDS research and new MDS medications available."

Your MDS website and newsletter helps the most with these issues. Please keep up the good work you do.

William H.

"What a wonderful surprise to receive such a complete information package on MDS"

I'm excited to pass it on to my mother. I appreciate your thoughtfulness in sending it and I know my mom will appreciate it as well. Would have been nice to receive this information from her doctor a long time ago. Thank you very much.

Cindy L.

"Just wanted to say thank you for the 100 Questions & Answers About MDS Book"

I'm putting together information about MDS for the BC Cancer Agency Patient Library here in Victoria. It's an excellent resource.

Pam W.

See BBoH on page 13



2016 RARE DISEASE DAY

Rare Disease Day in NJ

The New Jersey Rare Disease Alliance brought patient advocates and industry experts to Trenton on February 29, 2016 to mark *Rare Disease Day*. With a healthcare system that's often geared toward treating those with more common diseases, patients with rare ailments can struggle to get the attention necessary to develop treatments.

The NJ Rare Disease Alliance is a state-based coalition of different rare disease groups, of which the MDS Foundation is a member, hoping to find strength in numbers — hoping their combined lobbying might get the attention of the biotechnology and pharmaceutical companies with the potential to develop treatments.



Fantastic Day in Trenton. Some faces of Rare Diseases as they march to meet Senators and bring awareness!

Each individual rare disease group can lack power at the state level, but together, we are a force. When we are just too

small, we don't have a voice, ALONE WE ARE RARE — TOGETHER WE ARE STRONG!!!

Announcing Our New Social Media Strategist

The MDS Foundation is proud to announce that we recently added a new member to our team, Janna Pelle, as social media strategist.

Janna is a musician living in Brooklyn who has contributed to MDSF through a variety of musical endeavors from benefit concerts to bone marrow drives, to her album “The Show Must Go On”. Inspired by her father’s diagnosis with MDS, one hundred percent of the proceeds from her album sales are donated to the MDS Foundation towards research. In addition to her passion for music, Janna also has a degree in advertising from the University of Florida and a passion for raising awareness of MDS.

Hiring Janna seemed like a perfect fit, at a perfect time—in addition to having the necessary skill set for the position, Rare Disease Day was just around the corner, with this year’s theme being “Patient Voice.” To follow is an entry from Janna’s personal blog (jannapellemusic.tumblr.com), on her thoughts on music, her new position with MDSF, and her purpose in life since her father’s passing in July 2014.

Having a “Good Voice”

I’ve never thought of myself as having a “good voice.” I knew I had “my voice,” or a “distinctive voice,” but I didn’t ever really study voice, and have only cared enough to take a handful of vocal lessons.

I never would have expected it, but what made me realize I did indeed have a good voice wasn’t singing.

I recently started working at the MDS Foundation—the foundation working to cure the type of cancer my Dad had, a rare blood cancer and bone marrow failure disorder, also known as “pre-Leukemia.” My dad died in July 2014, and I have been continuing to be a dedicated contributor to the cause in his honor since his passing.

This year, the theme of Rare Disease Day was “Patient Voice: Making the Voice of Rare Diseases Heard.” That day at the

State House in New Jersey I launched the social media campaign for MDS I have been working on—sharing a short video and creating an interactive display. I was also asked to speak about my relationship with the Foundation, MDS, and my father, and play the song I wrote about his diagnosis.

The whole thing just seemed too perfect—the theme was “Patient Voice”—and it got me thinking about what it really means to have a “good voice.”

Even before I started as an official employee of the MDS Foundation, I have been working to make the “voice of rare diseases heard”. I recorded my EP, “The Show Must Go On” during my Dad’s struggle, with 100% of proceeds going to the MDS Foundation towards research. I made the album “pay what you wish,” and beyond exceeded my expectations of fundraising. One woman even purchased the album for \$999.99 (because that was the most digits bandcamp.com would allow).

I never expected to be asked to sing the *Star Spangled Banner* at the Be The Match Walk/Run 2 years in a row. Though I know what a challenging song this is (and I’m sure someone with professional vocal training could sing it better than me), it is an honor to be asked. I know they asked me because of my personal connection to the disease.

I never expected to be flown to Washington, DC, for the MDS Foundation’s International Symposium, to speak about my Dad, and perform “America The Beautiful” on a grand piano for the opening ceremony.

I also never expected my Dad to be taken from me due to a terminal illness, or for my lifelong friend Patrick Wanninkhof to be killed by a texting driver just a year later. What I did expect was that they would be around for the duration of my life.

But, because of them, I have learned to expect everything and nothing at the same time. Nothing shocks me anymore, whether good or bad.

This mindset has allowed me to realize that ANYTHING IS POSSIBLE.



I never thought I would be working for a non-profit cancer organization, literally employing all of my marketable skills as well as passions in the same place.

I never thought that my Dad would ever be able to help me get a job again—but he has.

And I never thought that anything but music would ever fulfill me. Working with the MDS Foundation has helped me realize that I do have a “good voice”—and I am using it in a variety of different ways. THAT, not just my music, is my true passion.

I want to have influence. I am good at spreading messages. Whether it’s with singing/music/performance, a social media campaign, a Facebook post, a flier, public speaking, writing. Today, I know I have a “good voice”—because I am using my voice for good. And I can change the world with it. I already have, but this is just the beginning.

Sometimes, though, I still need to remind myself to have a “Patient Voice.”

In honor of Rare Disease Day, with our new social media strategist’s help, we launched our very first social media campaign—the **#stoptheMaDnesS** campaign—to continue through the month of March. The campaign was also released in conjunction with the NCAA championship’s March Madness. In addition to raising awareness and encouraging people to **#stoptheMaDnesS** of MDS, the campaign also raised \$10,000 for the MDS Foundation—our pharmaceutical partner Celgene generously donated \$5 per tweet for the cause.

MDS PATIENT SUPPORT GROUPS & ADVOCATES SPREADING MDS AWARENESS AROUND THE WORLD



VIENNA, AUSTRIA



JAPAN



HERSHEY, PENNSYLVANIA



CHINA



UZBEKISTAN



ARGENTINA



UNITED KINGDOM

MDSF Chair, Dr. Stephen Nimer, visits an MDS Center of Excellence in China with our partner from Janssen China, Hu Jun.



SLOVENIA



MIAMI, FLORIDA

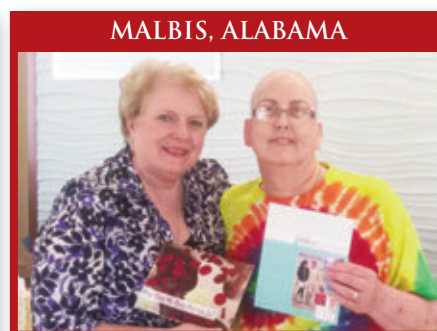
THANK YOU TO ALL FOR RAISING AWARENESS ALL OVER THE WORLD!



BRAZIL



TAMPA, FLORIDA



MALBIS, ALABAMA

LuAnn Stevenson, Southern Cancer Center and Ann King, MDS Patient, commemorating MDS World Awareness Day



LITHUANIA



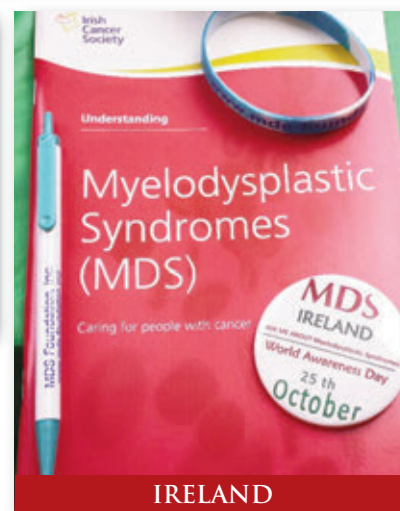
SINGAPORE



NEW YORK, NEW YORK



CANADA



IRELAND



LOS ANGELES, CALIFORNIA



GERMANY

PATIENTS & CAREGIVERS **LIVING** WITH MDS FORUMS

SPREADING THE NEWS WORLDWIDE

MARK YOUR CALENDAR FOR THE LOCATION NEAREST YOU!

PLANNED FUTURE MDS FORUM EVENTS FOR 2016

Don't miss out on these informative, FREE events.

Register Today

FOR OUR FREE EVENTS

MID-ATLANTIC & NEW ENGLAND

May 7, 2016

Lebanon, NH

September 10, 2016

Baltimore, MD

June 25, 2016

Boston, MA

October 29, 2016

*in honor of MDS World
Awareness Day*
New York, NY

MIDWEST/WEST

February 27, 2016

in honor of Rare Disease Day
Los Angeles, CA

October 29, 2016

*in honor of MDS World
Awareness Day*
Denver, CO

SOUTH

February 27, 2016

in honor of Rare Disease Day
Tampa, FL

June 25, 2016

Birmingham, AL

September 10, 2016

Dallas, TX

November 12, 2016

Miami, Florida

INTERNATIONAL

June 11, 2016

Copenhagen, Denmark



LEARN MORE AT:

www.mds-foundation.org/patient-and-family-forums/

Many patients and caregivers have never met another person diagnosed with MDS until they connect with them at one of our forums. If you've never attended one before, 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.

ONLINE EVENT: The AML Patient Experience



Did you know that acute myeloid leukemia (AML) is the most common type of acute leukemia in adults? Nearly 40,000 people are diagnosed with AML in the U.S. and Europe each year, and for those patients outcomes are typically poor. Despite dramatic advances in other blood cancers, AML has not seen a change in the standard of therapy in the past 40 years and new treatment options are desperately needed.

In recognition of AML Day, The Leukemia & Lymphoma Society (LLS), MDS Alliance and Patient Power invite you to join a panel discussion on:

- Why have an AML Awareness Day and why now?
- The AML patient experience
- Resources and support for AML patients
- A continued focus and dedication to AML research
- The importance of clinical trials & challenges in AML

Panelists Represent:



This activity is supported by Celgene and Agios.

WHEN: April 21, 2016
TIME: 11 a.m. ET / 5 p.m. CEST
REGISTER: www.bit.ly/AMLDay

**VIDEO AVAILABLE
ON OUR WEBSITE
www.mds-foundation.org**

Please note: This panel is not a forum to discuss specific drugs or treatment options or to receive or provide medical advice. If you have questions about your condition or treatment please contact your health care provider.

Our Daughter's Journey with MDS and HOPE

Joanne Curran

Kingston, Ontario, Canada

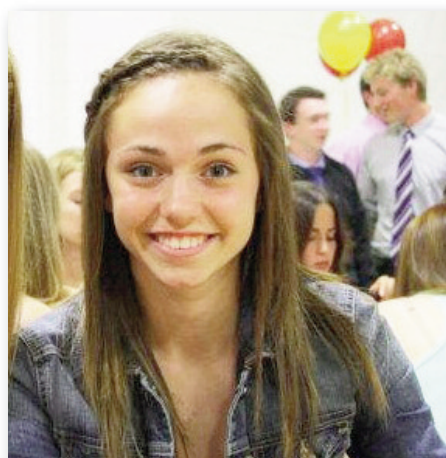
When our youngest daughter Mackenzie turned 16 on March 3, 2013 we had no idea her life was about to spiral out of control.

Mackenzie was a normal 16 year old teenage girl in Grade 10. She loved to play basketball, do hurdles in track and field, and hang out with her friends. Shortly after her birthday, while she was playing basketball she noticed she was extremely tired and could not keep up with her teammates. As an elite athlete, fatigue wasn't unusual for her so at first she brushed it off. The fatigue progressively became worse and worse, however, until one morning she was so tired she couldn't get out of bed. Her pediatrician had blood work done to rule out anything serious. Although her white blood counts were severely low, it was confirmed Mackenzie had a virus. She was told by her doctors there was nothing they could do to help her, she would just have to wait it out. She was told her body would eventually recover on its own. Mackenzie spent her final 3 months of Grade 10 exhausted, and missed numerous days of school. Thankfully, she was successfully able to complete Grade 10 as an honor student.

Five months passed and there was no change in her health. Eventually her red blood counts started plummeting.

In July of 2013, Mackenzie had her first of 4 bone marrow biopsies. On August 7, 2013 we received the devastating news that Mackenzie had myelodysplastic syndrome, monosomy 7. We were told MDS was extremely rare in children. Mackenzie's MDS was rapidly evolving into acute myeloid leukemia (AML). To prevent the AML from progressing further, Mackenzie needed an urgent bone marrow transplant. My husband, myself, and our 20 year daughter Kelsey were all tested but no one in our family was a match.

Word of Mackenzie's situation spread rapidly throughout our hometown of



Kingston, Ontario. People in Kingston and surrounding communities sprang into action by holding swab events in an attempt to find Mackenzie's match. They knew they may not be a match for Mackenzie, but could possibly match one of approximately 1000 people in Canada looking for a match at that time. We were told the frightening news that less than 50% of people needing a match actually find one.

The support our family received from our community was overwhelming. Numerous local high schools held swab events. All the players on a local Junior A Hockey team swabbed, and even our local university, Queen's University, got involved. Three teams — women's and men's basketball, and men's hockey — swabbed in support of Mackenzie.



We waited nervously for 3 months. Many prayers were sent up for Mackenzie and thankfully on November 7 we received amazing news that a match was found! Mackenzie's 4th bone marrow biopsy revealed 12% AML cells in her body. Time was running out as we were told the transplant would have to be cancelled if Mackenzie reached 20% AML cells.

One of the most frightening days in the MDS journey was meeting with Mackenzie's transplant doctor at The Hospital for Sick Children "Sick Kids" in Toronto, Ontario, and being told transplant was Mackenzie's best chance at survival — BUT there were life-threatening risks associated with it.

We left home on November 10, 2013, not knowing when we would be returning, and arrived at Sick Kids for 8 days of intense chemotherapy to prepare Mackenzie for the bone marrow transplant. She had never received chemotherapy before, so the pre-transplant days were extremely grueling, to say the least. As parents, it was excruciating to watch our child suffer.

On November 19, somewhere in the world, Mackenzie's donor was going through the process of donating his bone marrow stem cells. All we knew about him was that he was 23.

Mackenzie was transferred to an isolation room on the day of her bone marrow transplant, November 20, 2013. For 6 hours she received 3 bags of her donor's stem cells. Mackenzie was very sick following the transplant. Her liver and kidneys were attacked. It was awful to watch her suffer through mucositis, sleepless nights, and emotional despair. Despite all of this, for the most part her body tolerated the new cells quite well.

Five days after transplant she began losing her hair in large clumps. Twelve days after transplant she started to show signs of engraftment, meaning her donor's cell were beginning to work. Eighteen days after transplant we celebrated "Engraftment Day". Mackenzie was allowed to leave her



isolation room for a short period. She was extremely weak and even walking a few feet was a challenge.

On December 9, 19 days after her transplant, Mackenzie was able to move out of her tiny insolation room to a step down room. Her drive and determination throughout her transplant was incredible. Mackenzie made it her goal to make it home for Christmas. She had to begin eating before she would be allowed to leave the hospital. Mackenzie had lost 20 lbs — which was a lot for a girl who weighed only 110 lbs to begin with.

Mackenzie worked hard with her physiotherapist, and on December 18, 2013 she was released from the hospital. Christmas 2013 was by far one of our best Christmases ever!!

For the next 6 months Mackenzie was isolated in our home. She celebrated her 17th birthday in isolation and battled through some very low days, but for the most part she remained positive.



She was ecstatic to celebrate 6 months post-transplant away from our home with many friends, and even more ecstatic to celebrate 1 year post-transplant.

Mackenzie's 1 ½ year journey through MDS and a bone marrow transplant was challenging, to say the least.

One of the things that kept Mackenzie focused was her love of basketball. She had been playing competitive basketball since she was 8, and was determined to return to the basketball court one day — which she



did with her high school team in September 2014. Scoring her first basket that year was a moment we will never forget! Mackenzie played two seasons of basketball with her high school team and was ecstatic when they won the City Championships this past November 2015 — allowing them to compete in the Provincial Championships (aka State Championships).

Mackenzie has chosen to use her journey in a positive way and use her story to encourage people. She says: "If I can use my struggles to help inspire people, and eventually save another person's life... it's all worth it."



In addition to receiving stem cells from her donor through her bone marrow transplant, Mackenzie also received 27 blood transfusions. She decided to form a blood team, and is so thankful to all her

team members who have donated over 300 units of blood to date. She has also been enthusiastic to share her story by way of speeches and was thrilled when Canadian Blood Services asked her to be the face of their Holiday Campaign in December 2014. Part of this honor included sharing her story at Parliament Hill, Canada's "White House".

Mackenzie also received a Make-A-Wish trip. It was so impactful in her life she decided to pay-it-forward. Last summer, with the help of many, she was able to raise over \$15,000.00 — enough for 2 wishes — and is currently working on a 3rd wish.

She is often asked to share her story at charity events, and never turns down an opportunity.

This past December we received amazing news about Mackenzie's donor!! His name is Alexander and he is from Germany. As mentioned earlier, Alexander was only 23 when he selflessly agreed to save Mackenzie's life by donating his bone marrow. It is impossible to describe the emotions and genuine love and thankfulness we feel for Alexander. We have arranged to bring him to Canada in May to thank him in person for saving Mackenzie's life!

Mackenzie lost a year of school but will be graduating this June from Grade 12. She has already received early acceptance into a few Universities and plans to pursue a post-secondary education in the health care field. Today our family is extremely thankful to God, prayers, doctors, nurses, Alexander, blood donors and medical advances!

We hope Mackenzie's story has inspired you in some small way to have HOPE through your journey with MDS.



Connect[®] MDS and AML: The Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia (AML) Disease Registry

Celgene is researching the following objectives in MDS and AML patient populations:

- ❖ Current and evolving patterns for diagnosing, treating, and monitoring patients
- ❖ Outcome measures
- ❖ How routine practice compares to national treatment guidelines
- ❖ Treatment patterns and outcomes in patients with del(5q), with or without additional cytogenetic abnormalities
- ❖ Association of patient characteristics, treatment regimens and clinical outcomes with patient-reported Health Related Quality of Life (HRQoL) and economic outcomes
- ❖ Clinical outcomes based on treatment in patients with or without mutations
- ❖ Correlation between mutation detection/allele burden in bone marrow and peripheral blood samples
- ❖ Molecular and/or cellular marker's relation to prognostic classification, drug mechanism of action and clinical and treatment outcomes

Select eligibility criteria:

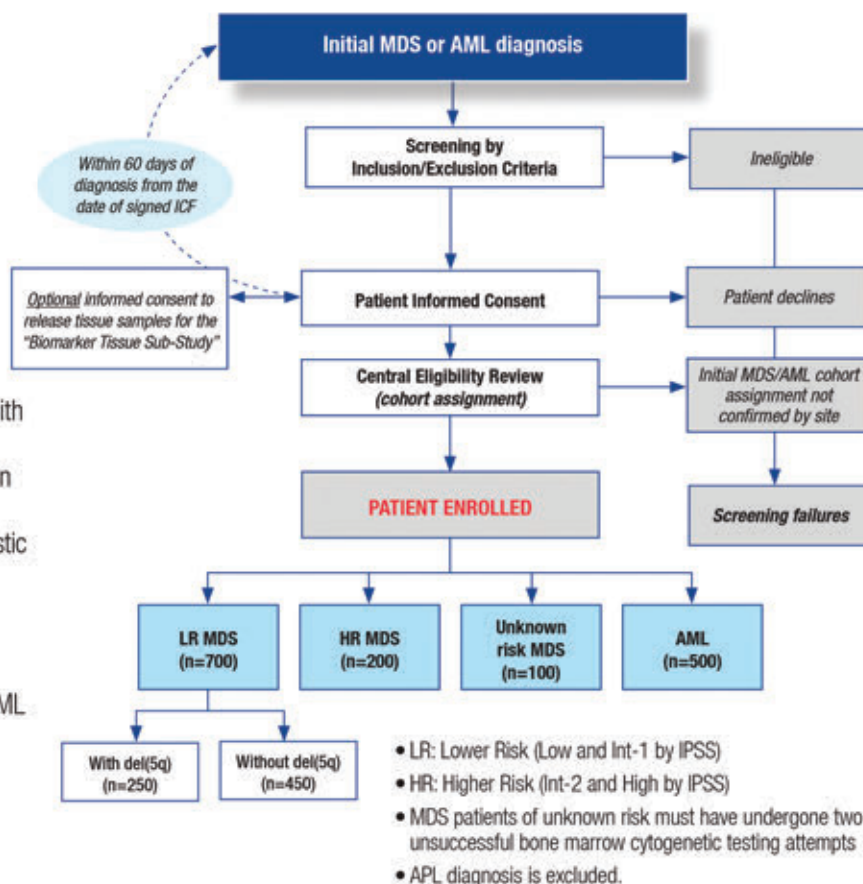
- ❖ Newly diagnosed,* primary or secondary MDS or AML
- ❖ MDS patients must be at least 18 years
- ❖ AML patients must be at least 55 years of age
- ❖ Patients must be willing and able to complete enrollment and follow-up HRQoL instruments, for which patients must be proficient in either English or Spanish

*To be considered "newly diagnosed," a patient's confirmed diagnosis must be made up to 60 days prior to the date of ICF signature.

Note: Concomitant patient enrollment in other studies is permitted.

Physicians – you could be an Investigator if:

- ❖ Your site supports clinical trials
- ❖ Your site sees at least 2 suspected MDS or AML patients per quarter



To learn more about this MDS/AML
Disease Registry Study, contact:
connectmdsaml-registry@celgene.com
(ClinicalTrials.gov Identifier: NCT01688011)



My Papa...

Amber Cornell
Fairfax, Virginia

My grandfather (Papa) was one of four children from a small town in central Maine. It is still to this day the type of small town where everyone knows everyone, where people still smile and wave in the streets, and where you can instantly feel comfortable with someone who you may have just met. His dad owned a tractor store and they had a small but tight-knit family. He was an athlete in high school playing four different sports and his name was always in the paper. Whether he threw the winning TD or scored a couple of free throws, people always knew “Dickie”. He met the love of his life when he was in second grade since she just so happened to be his best friend’s sister. They never dated in high school and she went off to nursing college and he went to Penn State for a semester before joining the Air Force. They often saw each other when she was home from school or when my grandfather was spending time with her brother. He once told me that the best thing he had ever done was accidentally propose to her and how surprised he was that she had said yes. But that was him, always humble in what he had and never took anything for granted. They were married for 51 glorious years before he passed away in September of 2014.

My Grandfather Has Always Been My Hero

It all started at a very young age, the first several years of my life we lived with my grandparents or lived within a 15-minute drive. At the time, I was the only grandchild who lived in the area and my parents took full advantage of “free” babysitting. There was even a scheduled date night on the calendar and every Thursday my grandmother or grandfather would pick me up from daycare and I would get to go to Gymboree, eat pizza and have them tell me my favorite bedtime



Richard and Carolyn, Maine 2011

story. Looking back, I know it was those special moments that allowed me to form a tight-knit bond with him that I will never lose sight of.

As I was growing up, I was fortunate to be able to see them often. When I was just turning 4 we ended up moving about 5 hours north of them to Jacksonville. We often spent holidays with my grandparents, we would drive down for weekend trips, and the most important tradition we kept was a weekly phone call to say hi. I remember being young and my mother would always call my grandmother on Saturday mornings. She would sit on the couch, talk to my grandmother for an hour or so and then it was finally me and my sister’s turn. I’d always ask to talk to Papa and we would chat for a couple of minutes about nonsense and then it would be my sister’s turn. Once I grew old enough my

few minutes turned into my own weekly phone calls on Sunday mornings with Papa. While the conversation may have evolved over time our weekly phone calls were something that never changed.

One weekend I was home from college and as I was getting ready to leave my mother told me she had something to tell me. I stopped packing and as she was explaining to me that my grandfather had been diagnosed with MDS, all I could think about was stopping what I was doing and calling him. I’ll be honest at the time I had no idea what MDS was or just how deadly this disease can be. Of course, in the true spirit of the internet age, my first task after my mom was done talking was google searching everything I could. I spent the next several weeks looking at what was being done in the field, treatment options, and just trying to understand all of the unknowns. I admit that I was certainly angry for some time, as most everyone feels after a loved one receives this news.

The worst part about everything was the dreaded question, just how long was he going to be around for? In February 2008 when we got this news he was already past the recommended age for a bone marrow transplant and so his options were certainly limited. He was over 60 and the doctor’s felt he would not be a good candidate for a transplant so it was never an option. The doctor’s gave him five years if we were



Richard Pratt TSgt, US Air Force 1965

lucky, and of course, that was with some very aggressive chemo and treatment plans. In true Papa fashion, he always kept a smile on his face, I never once heard him say “why me?” or ask himself what he could have done differently in life.

Throughout the next several years my grandfather lost weight, had chemo treatments and even was a part of one clinical trial. While the clinical trial wasn’t effective, the chemo had slowed but not stopped some of the advancement of his MDS. He also underwent one bypass surgery where they inserted stents for his heart, this was unrelated to the MDS but was certainly a scare with his condition. There were a few days after the bypass that he was unconscious for quite some time and we were afraid that it might be his time. My uncle flew down from New Hampshire, my parents drove down from Jacksonville and I’ll admit it was one of the more scary times. He ended up ok after that, he spent a week or so in the hospital but then he was able to go home. After the bypass, he also had a knee replaced. My grandfather had always had bad arthritis, that was one of the main reasons they ended up moving down to Florida. The cold winters of New Hampshire were never great on his joints and a much sunnier Florida was better for him. The knees followed with his arthritis and eventually both would need a tune up. Unfortunately, the MDS had weakened his immune system and he really could not keep any infections out of his knee. In 2012, he was in a wheelchair almost full time, while they continued to try and heal his knee. He would attend rehabs, spend weeks in and out of different facilities trying to heal his knee all the while his immune system was working overtime.

I think when I read of other patient stories about people battling for their lives, the people that are there every day are most often forgot about. My grandmother was always right by his side. Even now I sometimes forget that while he lived through the disease, so did my grandmother. When someone you love has

When I read of other people battling for their lives, the people that are there every day are most often forgot about.

MDS it certainly can take an effect on everyone in the family. I know sometimes that feels selfish to think or even say out loud, but it is a fact of the disease. My grandmother was no different. A nurse for 40 years, she was his nurse day one of his diagnosis til his last breathe. The last two years of his life were mostly spent in and out of hospitals, sometimes for weeks at a time. I think those were the hardest years, always uncertain of what was happening next and certainly more scares with his health than the first four. My grandmother was always so good about stopping her life, being by his side and holding a strong face. She was always encouraging him and rolling with whatever their day might bring them. I admire her so much every day for the pain she’s had to endure, that the rest of us didn’t always see firsthand.

The part I try and think of most often when I recall these memories, is just how happy he always was. Now don’t get me wrong, there was always an occasional day or time that he sounded tired or that there was a hint of pain in his voice. I think that comes with the territory and no matter how upbeat you can be, there are always hard days. We still had our weekly calls, we would talk about football or how my job was going, or what he heard at his men’s club meetings that week. It was never about how he hated being in these places or how he hated rehab. Even when I called while he was in the hospital he was always upbeat, chatty with the nurses or the people he shared rooms with. He always lit up a room he was in, no matter the situation, no matter the people he was surrounded with.

The last time I saw my grandfather everyone in my family showed up to surprise him. It was late August in 2014 and he was in a rehabilitation center still trying to get his legs moving. I was only able to drive down for two days but when I drove in I immediately went to see him. He was so excited to see everyone and the smile on his face that day was something I’ll never forget. I was fortunate enough to get to spend an hour or so alone with him. We watched part of a football game, which was one of his favorite things to do. We talked about the game, a little about my job and mostly kept it light. Right before I left I went over to him, hugged him tight, kissed him and told him how much I loved him. He laughed and said to me “How could anyone NOT love me?” and we chuckled for a second. It wasn’t an epiphany type moment, it wasn’t us crying together or talking about the end. It was just like us, chatting and living life in the moment, just like we did every Sunday.

Two weeks later they had moved him home with hospice, he was surrounded by my grandmother, my aunt and uncle, and my mother. I had spoken to him one other time and asked him if he thought I should come see him. He told me no, that he “had enough people fussing over him”. I could hear in his voice for the first time that he was truly tired and that he was ready. He had made it to the six-year mark, one more year than the doctor’s had originally given him. He passed the next morning.

It’s been a little more than a year since my Papa and I have had one of our Sunday calls. I’ll admit the first few months were hard and something that still chokes me up just thinking about. Then I always think how would he be in this situation? His calm and cool mannerism is something I’ve never been able to master but something that was always so easy for him. These are always traits that I strive to have, even though I will be the first to admit I’m terrible at. He always took life in stride, never dwelled on one particular event or problem that he encountered. He lived each day to it’s fullest, and for that, he’ll always be my hero.

Mam's Story

Sinéad Mahon
Dublin, Ireland

On the 15th of November 2009, my mam, Breeda Greaney, was Christmas shopping when she received the dreaded call from the hospital to come straight in. She was feeling tired all the time and out of breath. Routine blood tests showed low white and red blood cell count and she was admitted immediately to Tallaght hospital.

Mam's red blood cells were 7.3, her white blood count was so low that she was open to infection and her platelet count was 16. Following blood and platelet transfusions to improve her condition, mam, who was 56 at the time, was given a bone marrow biopsy to confirm the diagnosis. On the 2nd of December, mam's test came back inconclusive and this was the first time the Doctors mentioned MDS. After more tests were done, it was confirmed that mam had MDS on the 14th of December. Mam was then put on G-CSF for her white blood cells twice weekly and EPO for her red blood cells once a week.

Mam attended the day ward on a continuing basis needing blood transfusions. On the 23rd of December while getting a needed platelet transfusion, she developed a severe reaction requiring her to be hospitalised but thankfully mam was able to come home on Christmas Eve.

On the 6th of January 2010, mam had a repeat bone marrow test and from these results was referred to St. James's Hospital to talk about a bone marrow transplant. The haematologist consultant confirmed that mam was a candidate and there followed a stressful period of seeking a match for a bone marrow transplant match. All of mam's siblings got tested but sadly no one was a match. But, luckily, a series of intravenous drugs improved her white and red blood cell counts enough that she did not need a bone marrow transplant.

This pattern continued for the next three years but thankfully 2 years ago



Me and my sister

mam's doctors decided to reduce the growth factor and now mam does not need any treatment today. Five years later she is back at work and functioning normally. She still attends the hospital for check-ups and is thankful for her doctor, Prof. Dr. Helen Enright, consultant haematologist at Tallaght Hospital. Mam feels blessed that her treatment has worked and that she was lucky to live so close to the hospital and to be in her care.

During the early days of mam's diagnosis, our family experienced a rollercoaster of emotions. When mam was told she had myelodysplastic syndromes (MDS) we had, unsurprisingly, never heard of the condition nor could we find information about it in Ireland.

First we searched the internet which sometimes isn't so helpful when trying to find the best resources but fortunately we found a lot of information on American and UK websites. This was when we first contacted Sophie Wintrich of the MDS UK Patient Support Group and Audrey Hassan at the MDS Foundation.

I attended patient forums in London and Edinburgh to find out more. I set up a Facebook page in 2011, and sons and daughters of people with the condition made contact with us." Shortly thereafter, I founded MDS Ireland with the help of my family, Sophie Wintrich of the MDS UK Patient Support Group, Audrey Hassan of the MDS Foundation, and Prof. Dr. Helen Enright. In 2013, we had our first meeting for World MDS Day at Tallaght Hospital. The value of the group is to give people emotional support for a condition that most people haven't even heard of.

MDS Ireland meets with patients and family members three times a year and we have regular contact with people on a daily basis. We also do fundraising with the help of family members and patients. See mdsireland.com for more details. A free information booklet Understanding Myelodysplastic Syndromes (MDS) is available to order from the Irish Cancer Society at cancer.ie.



Me and my Mam, sister, family, friends, Prof Helen Enright, and day ward nurses – fundraising!

Caregiver Story

Brenda Ratliff
Bakersfield, California

It's almost ironic that when you've been married to someone for 44 years, they still try to tell you that all is well and they're feeling just fine — when you absolutely know it's not the truth. When God has woven your lives so closely together that you know that person's next heartbeat, words do not do much in convincing you of something that is contrary to what you see and feel.

I knew something was wrong when I had noticed for some time that my husband Steve was looking especially tired, pale and not always the happy go lucky man I knew so well. He is deeply committed to his family, an incredible father of three sons and loving grandfather of six grandsons, and a granddaughter, and the absolute love of my life. As a pastor, this outgoing people-person with a quick wit, undeniable laugh, and an incredible sense of humor, had grown quiet and it was scaring me. Last summer, he began experiencing severe leg cramps at night, interrupting his sleep, and it seemed to be getting worse. He tried all of the remedies that we knew of; salt water, walking, muscle massages — all without results. When he contacted his physician blood tests were ordered. He received a phone call instructing him to repeat the tests due to abnormal results. Several repeated blood tests and conversations later, he was referred to a local community based hematologist/oncologist in September for follow-up.

During the first appointment with the hematologist/oncologist, the physician made reference to what he felt the tests were leading him to suspect. This could be myelodysplastic syndrome, or MDS. My thought was myelo... what? He told us that there were other tests needed to rule out other possibilities, but if those results did not produce a diagnosis the next steps would be a bone marrow biopsy, and testing for MDS. Of course, as with most women, you do not mention in passing “could be’s or might be’s” and not expect a flood of questions,



and an “exactly how was that spelled”? He said “well let’s not go there IF we don’t have to.” Again, more tests and more waiting... but we also began researching MDS just in case. To be perfectly truthful, the information found on the web made the waiting even more difficult. As I read more and more, I questioned why this possible diagnosis even had to be mentioned?

A bone marrow biopsy was ordered for December, more tests were done, and again the wait. The physician said that he would be out of the office during the holidays, but that if the results were serious his colleague would call to let us know. Otherwise, Steve would be seen in January. As his appointment date neared, and there had been no calls, I felt comfortable that it couldn’t be “that serious”. But the phone call came, and Steve was told what his doctor had suspected — a diagnosis of MDS was confirmed. “Doctor will see you and explain the details during your scheduled appointment (10 days later).” More waiting, but this time we at least knew the name of what was causing the symptoms he had been experiencing.

Steve was diagnosed with MDS, and refractory anemia with ringed sideroblasts. The doctor discussed choosing the “just watch and see” approach for the time being. His recommendation — blood tests every three months with a follow-up depending on those tests. Immediately I thought “that’s it?” and “shouldn’t we be doing more to stop this?”. I am a firm believer in getting to the bottom of a problem, and finding a solution to fix it. Over the years I have heard my husband tell people during counseling, “None of us can be the fixer, that is God’s job. Our job is to be the prayers. He is in

control, and we have to trust that He has a plan for all of us, no matter what situations we might face.” Easier to hear that advice given to others — so much harder for me to apply to this situation. A close friend asked me how I was doing with all of this, and I told her that at times I felt like someone had hit me in the stomach and I just couldn’t seem to catch my breath.

I believe in being proactive and informed, so again I went online, contacted the MDS Foundation, and requested materials regarding MDS. Steve and I were astonished at the resources available, and especially at the quick response time in receiving the information. For once there was no waiting. A lot of answers to the questions that we had after his doctor’s appointment were found in the pages of *Building Blocks of Hope*.

We learned about the MDS Patient and Caregiver Forum in February in California and immediately reserved our seats. An opportunity to meet with others who also face this challenge, and hear from professionals researching and treating MDS. Amazing presentations were given by Dr. Gary Schiller and Erin Demakos, RN. The staff of the MDS Foundation was incredibly friendly and available. We heard discussions regarding the good AND the bad of this disease. There were interactions between those newly diagnosed, those that have dealt with MDS for many years, and open dialogue with the experts. I would encourage everyone to attend a forum. Follow-up since the event has been outstanding, reassuring us that we are not alone. There are people that have been where we are at now, and thankfully God has placed those people and the MDS Foundation in our lives as partners.

We did not choose this journey with MDS. IT chose us. Steve is due for his next blood test in a couple of weeks to see the comparison between January and now. There are still details that we do not know. Things are in the future that we cannot foresee, but I do know that God has lead us on many journeys over the years, and He will also take us through this one together — wherever it may lead.

The Accidental Survivor

Dianne Witter
Houston, Texas

I remember my first year of bone marrow failure in a haze; a series of images and feelings, a compilation of dreams, the main theme of which was, “This CAN’T be real!” When I slept — and I tried not to — I fell into disturbed dreams in which something nameless and terrifying was chasing me, or I was about to fall into a bottomless black pit. During the day, I often felt the same way, except the beast had a name.

Going back and reconstructing that time is like unraveling a ball of string that has gotten hopelessly tangled in the back of a junk drawer. When I take the end of the string and begin to pull... my life during that time—my twisted, messy, unexpectedly interesting life — falls out.

Sixteen years ago, I was a 40-year-old divorced mom who had just moved to Houston from Atlanta to live near extended family to have a support system in raising my then nine-year old son, Taylor. My cousin, who is like a sister to me, lived in Houston with her husband and two boys. Her parents, my aunt and uncle who had always been a second set of parents to me, lived in the same neighborhood. I had no idea at the time just how important that support system would turn out to be.

I’d been having some health problems for a couple of years, which had been diagnosed as fibromyalgia and chronic fatigue syndrome. In an effort to address some of the fatigue and brain-fog that is typical of fibromyalgia, I had left my job at a marketing agency to start my own freelance writing business. It had long been a dream of mine, and when an opportunity to work with several great clients on retainer arose, I leapt! And I loved it.

The Exhaustion Worsens

But even with a more flexible schedule, the exhaustion worsened. I think I realized,



as we were packing to move cross-country, that this amount of brain-dead exhaustion might not be normal, even for fibromyalgia. I made a mental note to look into it when we got settled in Houston.

Which I never did, and six months later, I wound up in the Emergency Room a few days after Christmas, with menstrual hemorrhaging from an unknown cause. I write about health care a lot and am a pretty informed patient. But I knew next-to-nothing about blood disorders. I thought I was having an exceptionally heavy period. But when I found I couldn’t even sit up without passing out, I knew it was time to go to the hospital.

It was well past time, actually. When the blood work results came back, I overheard the lab tech in the hallway telling the nurses my hemoglobin was “4 and some change” (4.2 to be exact). That meant nothing to me at the time, but from the nurses’ shocked expressions I knew that it was not good. (It’s hard now to imagine a time that I didn’t know that was about 1/3 as much blood as my body should have had.)

It was to be several months still before I had a diagnosis. For now, the focus was on getting the bleeding stopped. The first of many blood and platelet transfusions I would have that week was started. Fibroids were determined to be the probable cause of the bleeding, and I had an emergency hysterectomy.

It’s probably just as well that I didn’t know then what I know now about blood

disorders. I didn’t know how big the problem could be, so I wasn’t all that worried. Doctors and nurses word things so dispassionately that it can easily pass you by if you’re not ready to hear it.

When my doctor saw me on rounds the morning after surgery, she said something like, “The surgery went well. We did have a problem with getting the bleeding stopped, and that made for some tense moments.” A couple months later in a high-stress operating room scene on *Grey’s Anatomy*, one of the characters said, “Hurry; he’s going to bleed out on the table!”

Then, and only then, did it hit me in the head like a brick. All of it.

Mr. Toad’s Wild Ride

Thus I embarked on Mr. Toad’s Wild Ride. Once it became clear that my problem was much more than gynecological, I began a dizzying journey through doctors and medical centers and blood work, and transfusions and bone marrow biopsies. Though it wasn’t clear exactly what I had, it was clear that my body wasn’t making enough new blood cells to keep up with the demand. And that, I knew, was a really serious problem.

Another fortuitous circumstance of having just moved to Houston is that it’s home to MD Anderson, the nation’s top cancer center. With a tentative diagnosis of aplastic anemia, I was sent off to see a hematologist oncologist at MD Anderson’s Leukemia Center. I was lucky enough to get a doctor who was not only at the top of his field, but funny, sarcastic and irreverent (all qualities I prize in a person). He never minced words and I believe that, as much as anything, contributed to my surprising and unlikely longevity.

And it really was unlikely. All three blood lines were significantly suppressed and stayed that way for the next six years. I was transfusion dependent — sometimes needing them as often as every week. My platelet count had plummeted to around 10,000, leaving my blood unable to clot effectively and putting me at continual, serious risk of

bleeding to death from something as simple as a nosebleed — or from internal bleeding caused by nothing at all.

I lived on high alert, feeling like an anchor was suspended above my head by dental floss that could give way at any time. Having lived with the chronic fatigue of fibromyalgia for some time already, I didn't feel good, but I didn't feel much different than I usually did. But the psychological stress I was under was unbelievable. I was in the highest risk group, yet I looked completely normal — which was both a blessing and a curse. Traditional chemo drugs with extensive side effects weren't appropriate for my situation; in fact most of my treatment was simply supportive care. There weren't many options at that time.

During this time, I got a medical writing job at MD Anderson. I had had to resign most of my freelance clients when I first got ill, never knowing when I could work or what I could commit to, and was by this time financially pretty devastated, though family and a few close friends had kept me afloat for awhile.

Working where I was being treated just made sense — I was there a lot of the time anyway, and this netted me a salary, health insurance, and a view behind the scenes — a perspective few patients ever get. Co-workers knew I had some kind of blood disorder, but I was often sketchy on the details, not wanting to be seen as “dead man walking.” I was living, privately, at such high intensity and with such huge stakes, sometimes it was nice to be treated like just another co-worker.

Then I Got the Bad News

Things can always get worse. A bone marrow biopsy showed I'd developed chromosomal abnormalities that changed my diagnosis to myelodysplastic syndrome (MDS). With deletion 7q abnormalities and blood counts consistently dangerously low, I was in the worst prognosis category. I'd been living at high intensity for so long, I

The psychological stress I was under was unbelievable. I was in the highest risk group, yet I looked completely normal, which was both a blessing and a curse.

didn't think anything could really alarm me. But running across the term “average life expectancy of three to nine months” turned my blood to ice. (Well, what there was of it.)

Things got very real then. Time was on fire (to quote actor Evan Handler in his book “Time on Fire,” on his journey with leukemia). I was standing at the ledge of my life, looking over and trying to prepare myself for the leap. At that point, it gets really factual and common sense-oriented. There's no sappy music playing in the background and no Greek chorus singing of tragedy (especially if you haven't clearly communicated your situation to many people).

That's the thing about living with a fatal disease. It can be a simultaneously thrilling and terrifying roller coaster ride each day, but it's always authentic. There simply isn't time for anything else. I caught myself musing that there should be a Frommer's Guide to the Afterlife, and I wasn't completely kidding. It's not as scary when you're right there looking at it, and you can even make jokes.

What is scary is what's behind you, most importantly the son you've had an incredible bond with since birth. I was never afraid for myself, but leaving Taylor was unthinkable and unforgivable. (Yes, unforgivable. Don't argue with me.) What I wanted from life squeezed down into one tiny, but huge and desperate, plea — just let

me be there for him till he gets through high school. He was older than his years and he knew things were very serious, though we only touched on the topic of death a few times. I hated what my illness put him through, but I believe it was a big part of forging the responsible, caring man he is today.

And Then I Lived!

At that time, there were no FDA-approved medications for MDS, though one, Vidaza, was nearing final approval. I participated in a promising vaccine trial, but it wasn't helpful for me. I didn't qualify for other studies, so my doctor applied for permission to use Vidaza for me on compassionate use, as it was the only possibility for me.

I was on Vidaza for four cycles. By the fourth one, my counts pretty much bottomed out. They're expected to get lower at first, and then rise. There was no rising. My platelet count was down to 3,000 when we decided the Vidaza must be doing more harm than good and we should discontinue it. We'd wait a couple months for my counts to come back up, and “revisit our options.” (I don't think he really had a plan under “Options.”)

Several months went by, then several more. My counts came up by excruciatingly slow increments. But they came up. And then up some more. I remember the joy of hitting a landmark 20,000 platelets for the first time in six years. It seemed like such a wealth and promise of life, that number which had once seemed so daunting and fearsome. It really is all in the perspective.

I don't know why I'm alive. By every estimation, I should not be. At the time, studies of Vidaza said it could extend life an average of nine months. I think I officially went into remission close to a year after taking Vidaza.

And I've been in remission a full eight years now! I don't know how, and I don't know how long it will last. And honestly, I don't really care. I feel like I won the bone

marrow failure lottery. Or, as Taylor said, “Mom, you’re the luckiest unlucky person in the world!”

It’s not all easy. I’m on disability now and am pretty financially constrained. I still have fibromyalgia – which is much less dramatic, but causes more problems for me physically than bone marrow failure ever did. But having stood on the ledge and truly accepted the reality of a journey forward from this life — it doesn’t get more real than that, and that perspective gives me a peace and calm that some people search for their whole lives. When it comes down to it, not much of the stress we surround ourselves with in life really matters. And the few

things that do matter are really worth fighting for.

Taylor graduated from college last fall, and we celebrated with a mother-son trip to San Francisco. I’ve been there for the mundane things and for some really big, important things. He is 24 now, an adult, launching his own life and career. I’m so proud of him — I’m proud of us — for how we walked through fire and came out deeper, better people. Our relationship is one many parents would envy. My work here is done; anything else is cake.

But then again, sometimes I find myself daydreaming. Wouldn’t it be cool to be called “Grandma”? That would be cake and icing!



Taylor’s graduation from Texas State in August, 2015, was a doubly poignant milestone for us. Shortly afterward, we celebrated with a long-awaited trip to San Francisco and Napa Valley.



NEW RELEASE DATE DECEMBER 2016



A CHRISTMAS MOVIE ABOUT LOVE, FAITH, FAMILY, AND DEALING WITH MDS

At 17 years old, Angelica suffers from Myelodysplastic Syndromes (MDS) and receives chemotherapy as treatment. With her parents struggling with cancelled health insurance and her health declining, Angelica’s life depends on a bone marrow transplant. The search for such a donor is exceedingly difficult, however she needs a multiracial donor, making the search nearly impossible. Despite her severe condition, she finds friendship and love in 17 year old homeless runaway Sean. Together they witness a miracle proving that God works in mysterious ways. In this heartwarming Christmas film, Angelica will ultimately experience a love deeper than she could have ever expected.



We are thrilled to be in partnership with the MDS Foundation, to whom we are donating 5% of this campaign’s donations, and 5% of the film’s proceeds!

<http://www.yaleproductions.com/my-first-miracle.html>



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Talking with Others with Rare Diseases Empowers Patients

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

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

How long ago were you diagnosed with MDS?

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

How did you react?

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

It seems like being an active participant in your treatment is important to you. Do you see that in other MDS patients as well?

There are all kinds of people that make up this world of ours. I don't think that patients should feel like they are hostages.



Prue and Ray Malles

They shouldn't put their heads in the sand and never question anything. I want to know what my disease is and what the side effects of my treatment are.

Are there other ways that you have made your voice heard?

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

When you talk with other patients, what do you hope they take away?

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

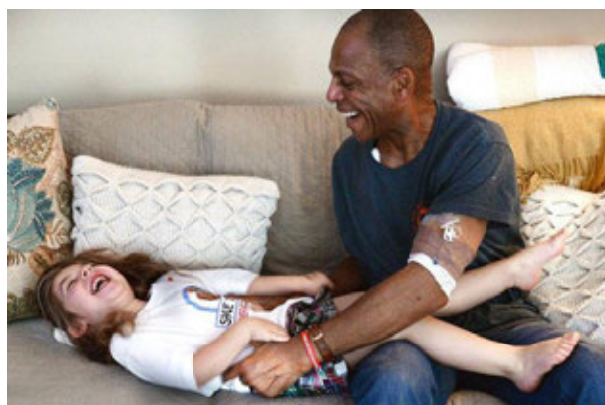
would like to speak with me. I answered their questions from my perspective, and they were very appreciative.

Since the Orphan Drug Act of 1983, the US FDA has approved over 500 treatments for rare diseases. What advice would you give to the FDA with regard to new treatments for rare diseases?

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

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

JUDGE CARL FOX, MDS SURVIVOR SPEAKS:



Here is something that likely will come as no surprise: most people tend to do what is easier, given a choice. This is not a judgment. The most motivated among us spend the majority of their lives performing simpler, rather than more difficult tasks. There is a misconception that something is inherently wrong with doing what is easy; that there is virtue in adversity. Somehow, we have come to believe doing that which is more difficult is more worthy of doing. Sometimes, this is true, but often it is just nonsense. By choosing the easier alternative, we can often achieve remarkable results.

Every 3 minutes in the United States, a person is diagnosed with a blood cancer – diseases including leukemia, lymphoma and multiple myeloma that affect blood cell production or function and damage the immune and circulatory systems. In 2015, almost 162,020 people in the United States were expected to be diagnosed with a blood cancer. This expectation does not include people who were expected to be diagnosed with other blood and marrow disorders like sickle cell anemia and MDS – my own diagnosis.

If I told you that addressing these challenges begins with a few cotton swabs and swabbing the inside of your cheeks, would you be surprised?

For many people suffering from blood-related illnesses, a bone marrow or peripheral blood stem cell transplant represents the best chance for their survival. While 30% of patients are able to find a matching donor in their families, according to Delete Blood Cancer DKMS, 70% of patients must rely on donations from strangers. These life-saving matches are made through donor registries and registering as a potential donor begins with swabbing your cheeks – a process that takes less than 60 seconds. **Think about it: at a time when there are countless causes competing for your attention — registering for a donor registry has the potential to save lives.**

Swabbing your cheeks and registering to be a bone marrow donor seems too easy, doesn't it? There is a cognitive dissonance here that is understandable. It is natural to assume that saving a life is prohibitively difficult and the notion that saving a life can begin with such a simple act conflicts with that assumption. Nonetheless, it is true and it is critical that people understand it.

Saving a life is a numbers game. For instance, think about the lottery. In Powerball, there are 292 million possible number combinations. If you buy one lottery ticket, your odds of winning the jackpot are close to zero. If you buy a million lottery tickets, representing a million different combinations of numbers, your odds of winning, though still slim, rise significantly. The fact is that finding a perfect match for a person in need of a life-saving donation is extremely rare because, on a genetic level, each of us is incomprehensibly unique. On average, there are more than 3 million differences between your DNA and the DNA of another individual. Much like playing the lottery, the odds of finding a donor match for a patient increase exponentially as more people register, which is why increasing the number of donors is crucial.

In the United States, this is particularly significant for **African Americans and other minority populations**. A match is most likely to come from a donor who shares the recipient's ethnic background. Unfortunately, African Americans and Latinos are severely underrepresented on donor registries – African Americans make up just 7% of the donor registry – greatly reducing their odds of finding matches. (It is worth noting that a bone marrow transplant is the only known cure for sickle-cell disease, a blood disorder that affects one in 500 African Americans in the United States.) When minority patients are diagnosed with serious and often life-threatening blood diseases and disorders, they are often surprised to learn their odds of receiving a life-saving transplant are extremely low because of the underrepresentation of minority donors in the donor registry. It is a sobering realization I assure you, speaking from experience.

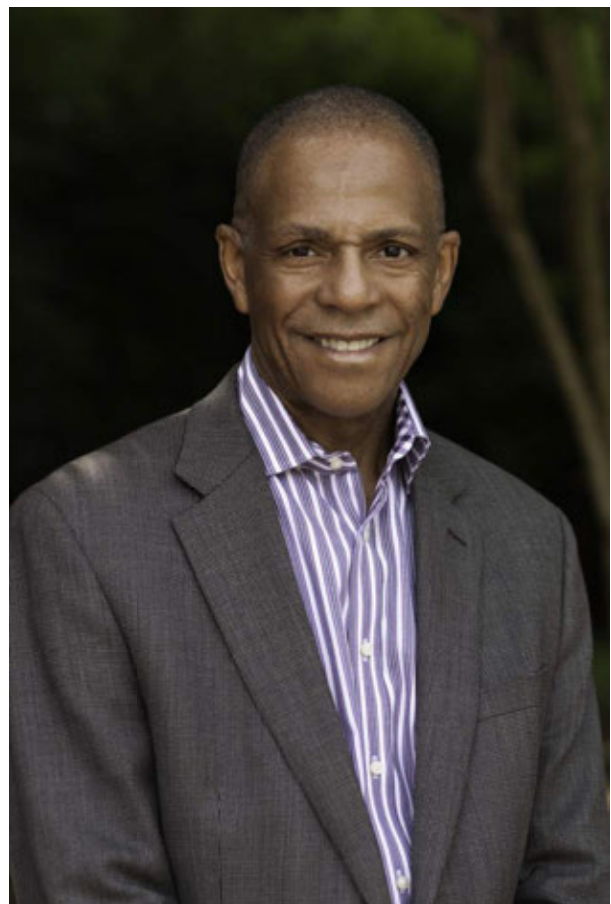
It is imperative that the African American and Latino communities recognize the scope and implications of this donor disparity and move toward an aggressive, proactive effort to close the gap by registering more donors, beginning with educating young people on this issue while they are in high school.

There are countless diseases, disorders and conditions we all hope will one day be eradicated. Despite our initial concern, many of us – white, black and brown alike – fail to act until we are personally affected. I certainly understand. Each of us has a hero inside us and we can become that hero so much easier than we realize. Saving a life can begin with a simple act. My challenge to you is to take 60 seconds today to do something easy, something remarkable, and something that could end up being heroic.

For more information or to send for a free swab kit, visit DeleteBloodCancer.org.

Carl Fox is a Superior Court Judge in North Carolina and holds the honor of being the state's first Black District Attorney. In August of 2015, Judge Fox was diagnosed with myelodysplastic syndrome and given three months to live. A successful transplant in September saved his life, and today, Judge Fox is living proof that donors save lives.

Statistic sources: <https://www.lls.org/http%3A/llsorg.prod.acquia-sites.com/facts-and-statistics/facts-and-statistics-overview/facts-and-statistics>



MDS CENTERS OF EXCELLENCE



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

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



ARIZONA

Mayo Clinic Hospital
Scottsdale, Arizona
Raoul Tibes, MD, PhD

The University of Arizona Cancer Center
Tucson, Arizona
Ravi Krishnadasan, MD, FACP

CALIFORNIA

Cedars-Sinai Medical Center
UCLA School of Medicine
Los Angeles, California
H. Phillip Koeffler, MD

City of Hope National Medical Center
Duarte, California
Stephen J. Forman, MD

Moore's Cancer Center at the University of California, San Diego
Rafael Bejar, MD, PhD
Peter Curtin, MD

Stanford University Medical Center
Stanford, California
Peter L. Greenberg, MD

UCLA Center for Health Sciences
Los Angeles, California
Gary J. Schiller, MD

University of Southern California Keck School of Medicine
Los Angeles, California
Casey L. O'Connell, MD

COLORADO

University of Colorado School of Medicine
University of Colorado Cancer Center
Aurora, Colorado
Daniel Aaron Pollyea, MD, MS

CONNECTICUT

Yale Cancer Center/Smilow Cancer Hospital
Yale University School of Medicine
New Haven, Connecticut
Steven D. Gore, MD

FLORIDA

All Children's Hospital
St. Petersburg, Florida
Gregory Hale, MD

Mayo Clinic
Jacksonville, Florida
James M. Foran, MD
Alvaro Moreno-Aspitia, MD

Moffitt Cancer Center
Tampa, Florida
Alan F. List, MD

Sylvester Comprehensive Cancer Center
University of Miami Miller School of Medicine
Miami, Florida
Stephen D. Nimer, MD

University of Florida Shands Hospital
Gainesville, Florida
Christopher R. Cogle, MD

GEORGIA

Emory Winship Cancer Institute
Emory University School of Medicine
Atlanta, Georgia
Amelia Langston, MD

The Blood and Marrow Transplant Program at Northside Hospital
Atlanta, Georgia
Asad Bashey, MD

ILLINOIS

Loyola University Chicago Cardinal Bernardin Cancer Center
Maywood, Illinois
Scott E. Smith, MD, PhD

Robert H. Lurie Comprehensive Cancer Center of Northwestern University
Feinberg School of Medicine
Chicago, Illinois
Olga Frankfurt, MD

Rush University Medical Center
Chicago, Illinois
Jamile Shammo, MD

University of Chicago Medical Center
Chicago, Illinois
Richard A. Larson, MD

INDIANA

Indiana University Simon Cancer Center
Indianapolis, Indiana
Larry Cripe, MD/Hamid Sayar, MD, MS

MARYLAND

Johns Hopkins University School of Medicine
Baltimore, Maryland
Amy Elizabeth DeZern, MD

University of Maryland Greenebaum Cancer Center
Baltimore, Maryland
Maria R. Baer, MD

MASSACHUSETTS

Children's Hospital Boston
Boston, Massachusetts
Inga Hofmann, MD

Dana-Farber Cancer Institute
Boston, Massachusetts
Richard M. Stone, MD
David P. Steensma, MD
Benjamin Ebert, MD, PhD

Massachusetts General Hospital Cancer Center
Boston, Massachusetts
Timothy Graubert, MD

Tufts University School of Medicine
Tufts Medical Center
Boston, Massachusetts
Kellie Sprague, MD

MICHIGAN

Barbara Ann Karmanos Cancer Institute, Wayne State University
Detroit, Michigan
Charles A. Schiffer, MD

William Beaumont Hospital Cancer Center
Royal Oak, Michigan
Ishmael Jaïyesimi, DO

MINNESOTA

Mayo Clinic
Rochester, Minnesota
Mark R. Litzow, MD

University of Minnesota Medical Center, Fairview University of Minnesota Medical School
Minneapolis, Minnesota
Erica D. Warlick, MD

MISSOURI

Washington University School of Medicine
Siteman Cancer Center
St. Louis, Missouri
John F. DiPersio, MD, PhD

NEBRASKA

University of Nebraska Medical Center
Omaha, Nebraska
Lori Maness, MD

NEW HAMPSHIRE

Dartmouth-Hitchcock Medical Center and Norris Cotton Cancer Center
Lebanon, New Hampshire
Kenneth R. Meenan, MD

NEW JERSEY

The Cancer Center of Hackensack University Medical Center
Hackensack, New Jersey
Stuart Goldberg, MD

NEW YORK

Albert Einstein Cancer Center/Albert Einstein College of Medicine of Yeshiva University
Bronx, New York
Amit Verma, MD

Columbia University Medical Center
New York, New York
Azra Raza, MD

Memorial Sloan-Kettering Cancer Center
New York, New York
Virginia M. Klimek, MD

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

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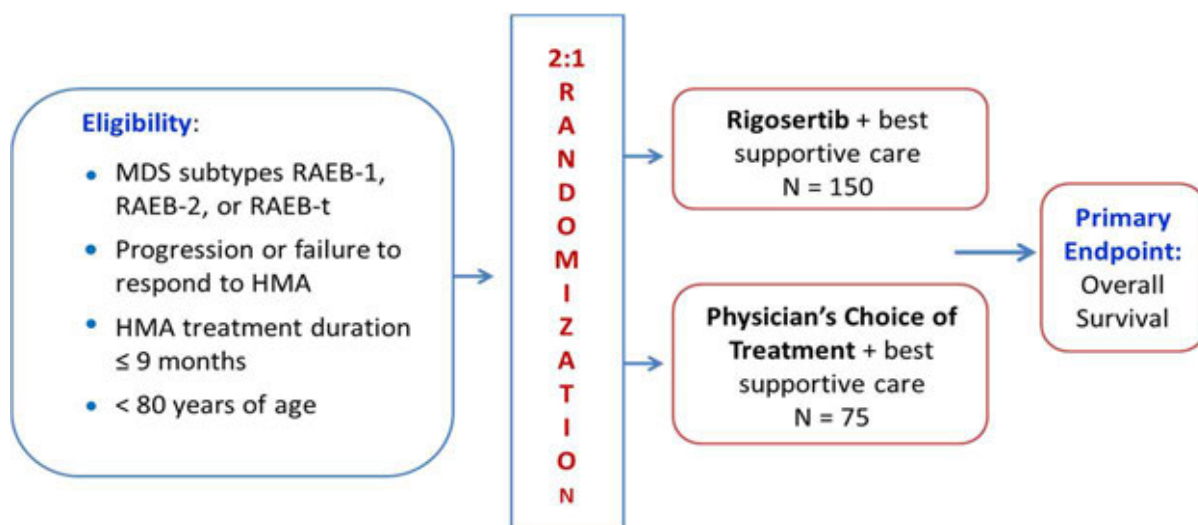
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For MDS Patients
After HMA failure

INSPIRE Phase 3 Trial

International **S**tudy of **P**hase III **I**ntravenous **R**igos**E**rtib

A Phase III, International, Randomized, Controlled Study of Rigosertib versus Physician's Choice of Treatment in Patients with Myelodysplastic Syndrome after Failure of a Hypomethylating Agent



Primary Endpoint: Overall survival

- in the intention-to-treat population, or
- in the subgroup with very high risk per the Revised International Prognosis Scoring System (VHR-IPSS-R)

Locations:

- More than 100 trial sites in North America, Europe, Japan, Australia and Israel

For additional information on this study, please call the INSPIRE help line at 1-267-759-3676 or visit www.clinicaltrials.gov, identifier: NCT02562443

Rigosertib is an investigational agent and is not approved by the FDA or other regulatory agencies worldwide as a treatment for any indication.

Rigosertib prior clinical data: Lancet Oncology

Rigosertib mechanism of action: Cell



San Diego-based La Jolla Pharmaceutical Company is currently seeking qualified patients for consideration in their trial in Patients at Risk for Iron Overload using its novel formulation of hepcidin (LJPC-401).

Hepcidin, an endogenous peptide hormone, is a naturally occurring regulator of iron absorption and distribution. Manipulation of hepcidin has the potential to prevent excessive iron accumulation in vital organs, such as the liver and heart, where it can cause significant damage and even result in death. La Jolla is developing LJPC-401 for the potential treatment of iron overload, which occurs as a result of diseases such as myelodysplastic syndrome (MDS), beta thalassemia, hereditary hemochromatosis, and sickle cell disease (SCD).

LJPC-401 has been shown to be effective in reducing serum iron in preclinical testing involving animals. La Jolla's Phase 1 clinical trial of LJPC-401 in patients at risk of iron overload is currently ongoing. Phase 1 trials are intended to evaluate safety and potential dosing requirements and are not intended to evaluate efficacy.

La Jolla is currently seeking patients with conditions putting them at risk for iron overload for a Phase 1 clinical trial at study centers in the United States.

Enrollment criteria include the following:

Patients with any of the following disorders:

1. Refractory or hemolytic anemia of any type. Examples include, but are not limited to, MDS, hemoglobinopathies, sideroblastic anemia, and congenital anemias

AND

Who have been transfused with at least 2 units of blood over the past two (2) months, OR 4 units of blood over the past six (6) months, OR received any form of iron chelation therapy over the past six (6) months, OR have a serum ferritin > 1,000 µg/L.

2. Patients with hemochromatosis that require phlebotomy at least once every two months OR have received iron chelation therapy in the past six (6) months.

Patients 18-85 years of age, inclusive.

Anyone wishing more information about this study may contact the following clinical trial sites:

Cincinnati, OH @ Jennifer Palmer – (513) 721-3868

<http://www.ctifacts.com/research-center.aspx>

San Diego, CA @ Ruth Garcia – (877) 500-3788

<http://estudysite.com/studies>

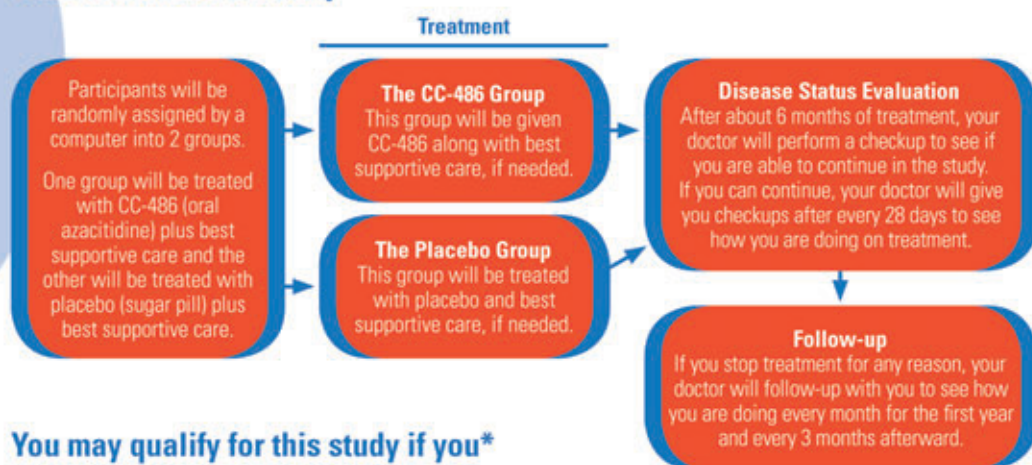


Do you have myelodysplastic syndromes (MDS)? You may be eligible for this clinical study

Announcing the QUAZAR Lower-Risk MDS Study

QUAZAR Lower-Risk MDS is a study for people with MDS who need blood transfusions due to low red blood cell counts (called anemia) and low platelet counts (called thrombocytopenia).

The QUAZAR MDS Study



You may qualify for this study if you*

- Are age 18 years or older
- Have been diagnosed with MDS
- Have low red blood cell counts and are dependent on blood transfusions
- Have low blood platelet counts

You may not be eligible for this study if you*

- Have had previous stem cell transplants
- Have been treated with VIDAZA® (azacitidine for injection) or DACOGEN® (decitabine for injection)

* Additional criteria apply.

DACOGEN is a registered trademark of Eisai Inc. and Janssen Pharmaceuticals, Inc.
VIDAZA is a registered trademark of Celgene Corporation.

For more information about this study

- Call 646-307-8079 or toll-free at 866-743-9791
- E-mail QuazarMDSstudy@emergingmed.com
- Scan the QR code



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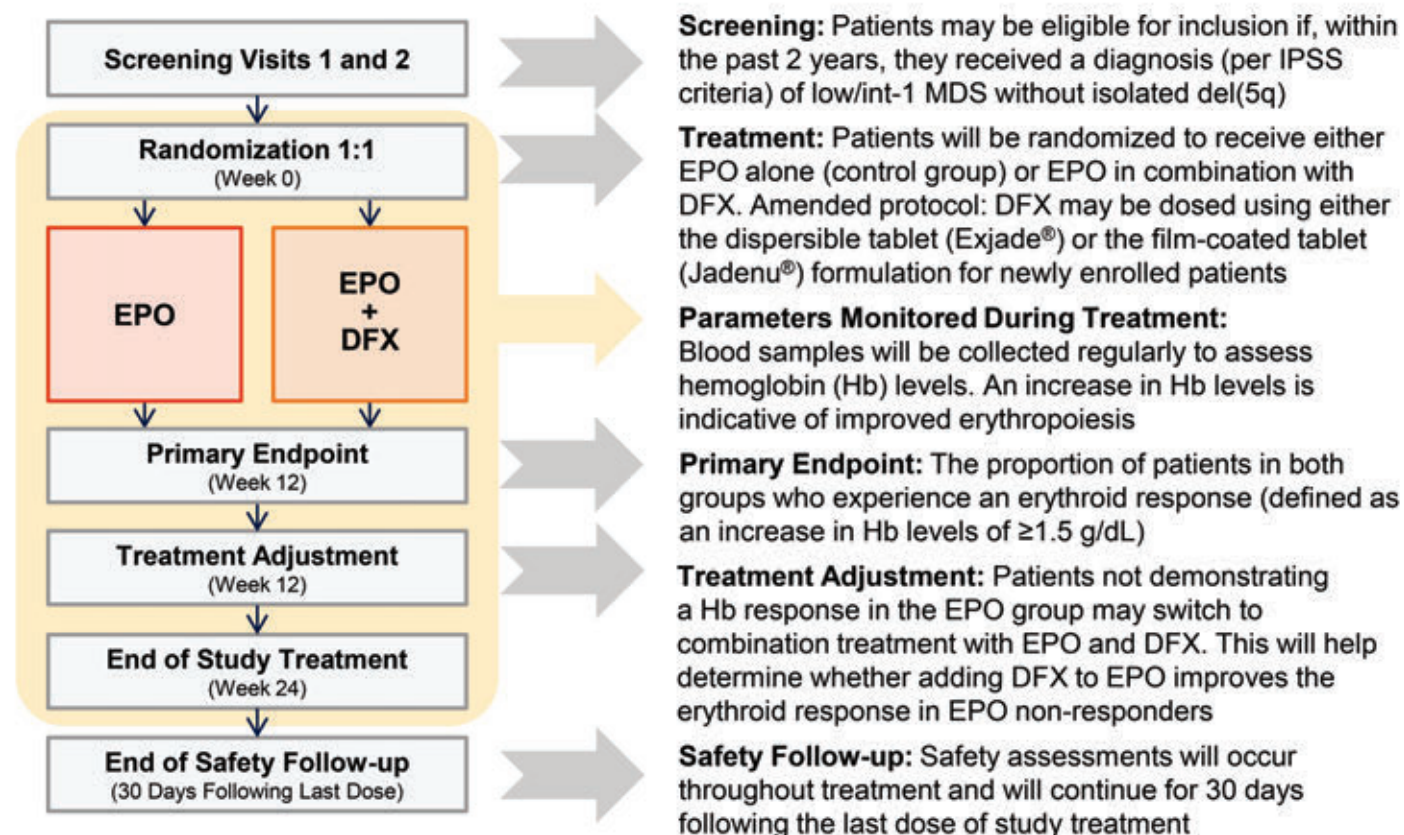
Overview

KALLISTO is an open-label, Phase II, pilot study of deferasirox (DFX) and erythropoietin (EPO) versus EPO alone in patients with lower-risk myelodysplastic syndromes (MDS).

EPO is a hormone that stimulates red blood cell production (erythropoiesis) in the bone marrow. Treatment with EPO can increase healthy blood cell counts in patients with MDS. DFX – an orally-administered iron chelator – may act synergistically with EPO to increase the production of healthy red blood cells.

The objective of KALLISTO is to test whether this potential synergy of DFX and EPO (versus EPO alone) improves erythropoiesis in patients with lower-risk MDS (low/int-1 per International Prognostic Scoring System [IPSS] criteria).

Study Schedule



To learn more about KALLISTO, call Novartis Pharmaceuticals (1-888-669-6682) or contact your local Novartis Medical Science Liaison (ClinicalTrials.gov Identifier: NCT01868477)





NEW CLINICAL STUDY: IMerge Lower-Risk MDS

Now Enrolling: NCT02598661

Janssen Research & Development, LLC is currently recruiting patients for a Phase 2/3 clinical study referred to as "IMerge", with the study drug Imetelstat, which is a first-in-class telomerase inhibitor. With its novel mechanism of action, Imetelstat may provide clinical benefit to MDS patients. In this study, Imetelstat is administered as a 2-hour intravenous infusion every 28 days.

IMerge is a study for people with MDS who need blood transfusions due to anemia (low red blood cell counts). People with low or intermediate-1 risk MDS that has relapsed or is refractory to Erythropoiesis-Stimulating Agents (ESAs) treatment may participate in the study. This study is being conducted at multiple hospitals and institutions around the world, in approximately 80 sites globally.

You may qualify for this study if:

- You are 18 years of age or older
- You have been diagnosed with MDS
- You have Low risk or Intermediate-1 risk MDS based on the International Prognostic Scoring System (IPSS), which is a standard prognostic tool
- You are considered to be red blood cell (RBC) transfusion dependent
- Your doctor does not think that ESAs have or will further help your anemia

If you are a patient with MDS, or if you are a physician/health care provider and would like to refer a patient for enrollment into this clinical study, please visit

www.clinicaltrials.gov (NCT02598661)

VISIT US ONLINE



MDS FOUNDATION HEADQUARTERS

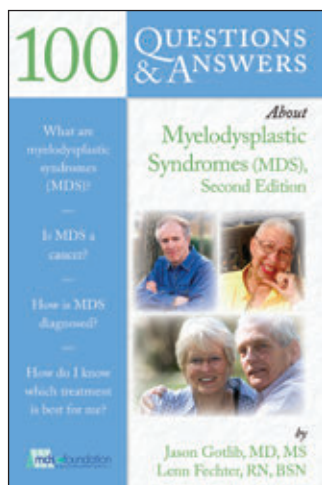
Contact Us:

800-MDS-0839 (*within US*)
609-298-1035 (*outside US*)
609-298-0590 (*fax*)

Or Write:

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

HAVE QUESTIONS ABOUT MDS?



SECOND EDITION

100 QUESTIONS & ANSWERS ABOUT MYELODYSPLASTIC SYNDROMES

This book is available free of charge through the MDS Foundation and is an invaluable resource for all MDS patients!!

It has been updated with essential and practical answers to 100 of the most frequently asked MDS questions — including available treatment options, what to expect during and after treatment, quality of life, sources of support, and much more.

Please contact the Foundation at 1-609-298-1035 or email jbutchko@mds-foundation.org for your complimentary in print or digital format version.

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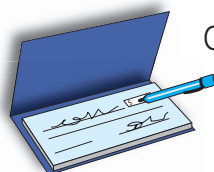
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Become a Member, attend a Patient Forum, or join our Online Discussion Site.



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