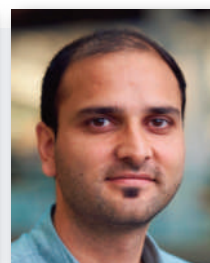


## MDS NEWS HIGHLIGHTS

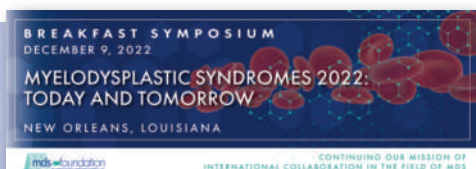
### FROM THE GUEST EDITOR'S DESK

■ **INFLUENTIAL ROLE OF BONE MARROW MICROENVIRONMENT IN MDS**

Presented by: *Dominique Bonnet, PhD and Syed Mian, PhD*  
 Haematopoietic Stem Cell Laboratory  
 The Francis Crick Institute, London, UK



### PLAN TO ATTEND



**ASH 2022:  
 MDS FOUNDATION SYMPOSIUM**  
 December 9, 2022, New Orleans, Louisiana

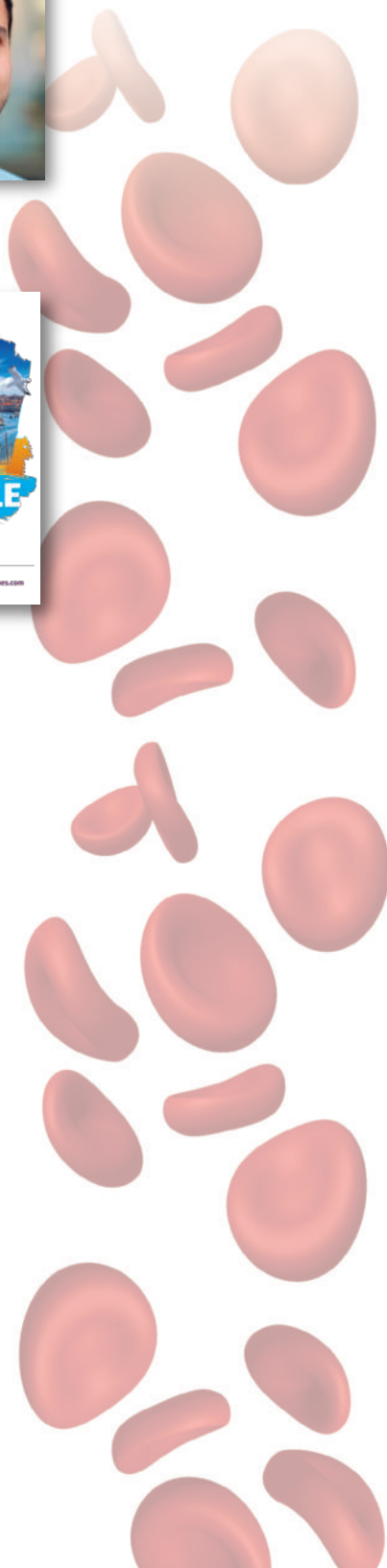
### 17TH INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES

3–6 May 2023  
 Marseille, France

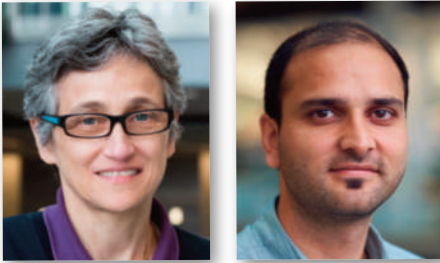


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### INFLUENTIAL ROLE OF BONE MARROW MICROENVIRONMENT IN MDS



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#### INTRODUCTION

Myelodysplastic syndromes (MDS) are a collection of clonal hematopoietic stem and progenitor cell (HSPC) disorders characterized by the presence of persistent cytopenia as well as morphological dysplastic changes involving one or more cell lineages in the bone marrow.<sup>1,2</sup> Over the last decade significant progress has been made in dissecting the MDS disease heterogeneity by the identification of somatic gene mutations in core cellular pathways such as DNA methylation, chromatin modification, RNA-splicing, signal transduction, cohesion regulation and some transcription factors.<sup>3-6</sup> Like any other cancer, emerging evidence suggests that combination of recursive rounds of positive clonal selections over the lifetime, plays a central role in determining the landscape of the disease. In MDS, clonal dominance is reported to be dominant at the time of diagnosis, however the mutational landscape does not entirely explain the conspicuous advantage gained by these malignant HSPCs. Notably, these malignant clones continue to coexist alongside healthy HSPCs, which are somehow kept under controlled suppression. Recent evidence has suggested that MDS can be preceded by an asymptomatic phase characterized by clonal expansion of HSPCs, a term coined as 'clonal hematopoiesis of indeterminate potential' (CHIP).<sup>7-10</sup> Although, CHIP itself is not considered malignant, at least for now,

however, population-based studies have demonstrated a strong association with developing myeloid cancers and various autoimmune diseases.<sup>11</sup> Interestingly, the risk of disease progression and transformation from MDS to AML, is significantly heterogeneous among MDS patients,<sup>2</sup> even though, the precursor states of MDS and AML are associated with similar mutations conferring a clonal advantage. Therefore, the probability of disease transformation might also be dependent on the external factors that are permissive for clonal expansion. The bone marrow tissue resembles a cellular 'megalopolis' or 'supercity', where various regions ('specialized niches') are connected via a complex vascular network.<sup>12,13</sup> This could be a key mediator that is providing a 'fertile inflamed' milieu where interactions between these components result in disease homeostasis and eventually progression. A multitude of different cell types including osteoclasts, osteocytes, adipocytes, sympathetic neurons, non-myelinating Schwann cells, and the largest among all endothelial cells (ECs) and mesenchymal stromal cells (MSCs), along with the immune-related cells co-exist to form bone marrow microenvironment (BMME). These cells distinctively organized in anatomical regions (i.e. endosteal niche, perivascular niche, arteriolar niche, central medullary niche) in the BMME have a specialized role in maintaining quiescence, homing and mobilization of the HSPCs. These highly regulated processes are driven via direct cell-cell contact as well as mediated via signalling molecules secreted by the cells in the BMME.<sup>12</sup>

#### BMME: A FERTILE MILIEU

The role of BMME in development of MDS is starting to emerge, however, significant amount of evidence already exists pointing towards its role in other cancers.<sup>14</sup> For example, studies from leukaemia's and other solid cancers suggests the major components of microenvironment (such as endothelial cells, ECs; mesenchymal stromal cells, MSCs)

could be key mediators that are providing a 'fertile milieu' where interactions between the malignant cells and these niche components result in disease homeostasis (**Figure 1**). The evidence for the disruption of the BM architecture in MDS emerged in early 90s that coined a term "abnormal localisation of immature precursors" (ALIP) which turned out to be a histopathological hallmark of MDS. The term itself describes the abnormal localisation of HSPCs in the BM interstitium, rather than paratrabecular endosteal niche as typically observed in healthy BM.<sup>15,16</sup> Increased BM vascular density that correlates with increased BM myeloblasts and advanced MDS disease has been also reported.<sup>17-19</sup> Further evidence for the involvement of ECs in MDS has been suggested by a study where EASIX (Endothelial Activation and Stress Index, [creatinine × LDH]/platelets), an endothelial dysfunction related biomarker, provided an independent predictor of survival in low-risk MDS patients.<sup>20</sup> The molecular defects in endothelial progenitors that have been reported over the years in MDS appears to be intrinsic in nature, since the transcriptomic and epigenomic landscape seems to be distinct, with enhanced autophagy and lysosomal degradation features along with aberrant HSPC supporting ability.<sup>21-23</sup> For example, angiogenic growth factors such as VEGF, Ang-1, angiogenin, FGF- $\beta$ , HGF are all significantly increased in MDS and some other myeloid cancers.<sup>24</sup> Increased proliferation of MDS progenitor cells have been linked with VEGF dysregulation that itself promotes both paracrine signalling to mediate BMME remodelling. In fact, VEGF induction led to the increased vascular permeability in sinusoidal ECs, therefore causing increased cell cycle, migration and differentiation of HSPCs, with myeloid bias and increased apoptotic rate. Stimulation of VEGF has also been linked with increased colony forming ability of primary cells derived from high-risk MDS patients.<sup>23-25</sup> Some of these cellular phenotypes are hallmarks of hematopoiesis in MDS, and at least partially provides a biologic rationale for ALIP and its

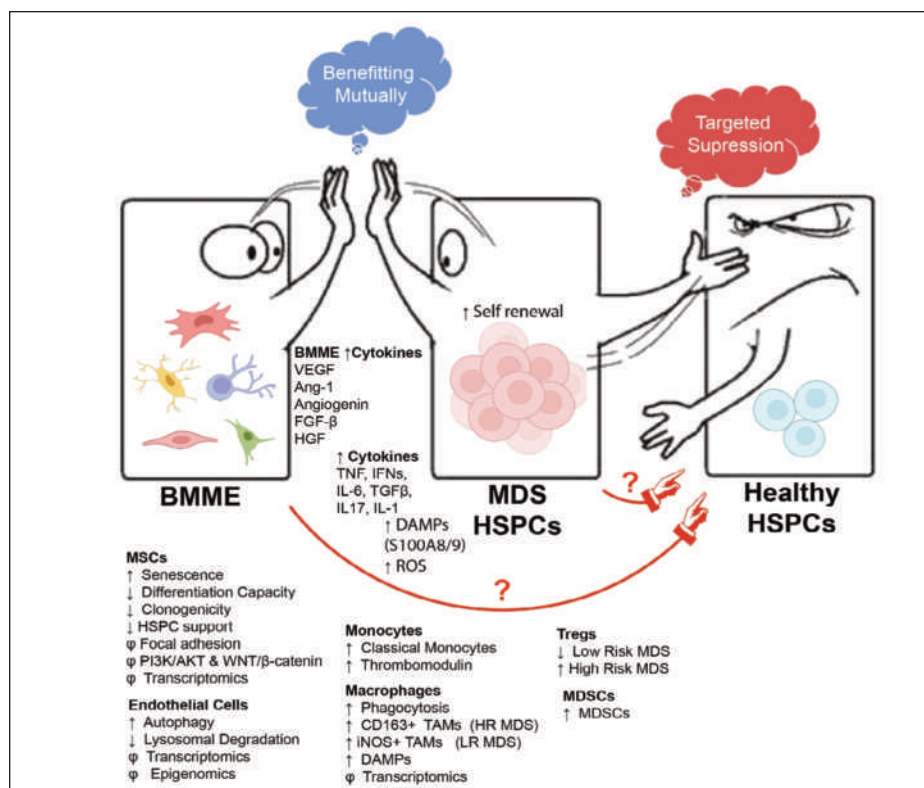
clinical association with adverse prognosis observed in high-risk MDS. Further studies are urgently needed to conclusively ascertain if the ECs in the BMME forming the vascular milieu are themselves dysfunctional, and if any, the role they play in driving myelodysplasia.

MSCs in the MDS BMME (**Figure 1**) have been suggested by some to be clonal in origin, however, more work needs to be done to establish this notion. These MSCs derived from the BM of MDS patients have reduced clonogenicity and increased senescence, especially in high risk disease such as refractory anemia with excess blasts. Defects in adipogenic as well as osteogenic differentiation potential have also been well documented. Contradictory reports about the presence of 'myeloid related-gene' mutations

or chromosomal abnormalities in these MSCs exist that has also raised questions about the clonal nature of these MSCs.<sup>24,26-30</sup> Despite the altered transcriptome landscape of MDS MSC themselves is well established, the molecular mechanism by which MSCs contribute to the disease in the MDS BM remains to be established. Over the years, observations relating to MSCs in MDS patients, such as altered expression of adhesion proteins, focal adhesion kinases, PI3K/AKT signalling, and WNT/ $\beta$ -catenin signalling, have been correlated with the increase in senescence and dysfunctional differentiation observed.<sup>24</sup> Furthermore, critical contributions of the BMME to the MDS has further been suggested by the inability of human MDS stem cells to propagate in a cell-

autonomous manner in immunodeficient mouse models.<sup>31,32</sup> Recently, ectopic humanized niches in immunodeficient mouse models were shown to successfully establish and maintain MDS clonal architecture. In this report, MDS stem cells were able to maintain the clonal landscape as well as engraft not only in the autologous humanized niches but also in allogenic healthy humanized ectopic niches. This humanized niche in-vivo model system enabled a balanced hematopoiesis output where HSPCs were able to differentiate to mature cell types including myeloid cells, monocytes, macrophages, erythroid cells and neutrophils. Interestingly, MDS HSPCs were able to migrate and home to other ectopic niches that were pre-seeded with human MSCs. Contrary to healthy donor stem cells, no migration of MDS stem cells was observed to the other murine hematopoietic tissues or even to the ectopic niches seeded with murine MSCs.<sup>33</sup> Further studies are required to understand the dynamics of such preferential migration and whether this is associated with normal HSPC behaviour or rather, point to a more aggressive MDS initiating cell intrinsic properties, driven or not by external niche factors.

Another question that needs to be addressed, is whether the BMME has any influence, directly or indirectly, on the residual healthy HSPCs (**Figure 1**), present in the BM of MDS patients. A recent study has reported that healthy donor HSPCs that were co-cultured with MDS MSCs resulted in a failure for these HSPCs to engraftment in immunodeficient mouse recipients.<sup>34</sup> This observation raises the question, whether MSCs themselves are dysfunctional in MDS or whether this is due to a secondary effect, induced by the MDS HSPCs, on MSCs which could not only persist for longer duration in MSCs but can also be transmitted to healthy HSPCs. The capability of MSCs to respond to the external cues based on cell-cell interactions have not only firmed a longstanding view of their functional plasticity properties, but also opened new questions about the imprinting of memory in MSCs that needs to be further studied. Remodelling of



**Figure 1. Interactions of BM microenvironment cells in MDS.**

MDS HSPCs acquire increasing self-renewal capacity with a high dependence on cells in the BMME. MDS HSPCs coexist alongside normal wildtype healthy HSPCs, which are suppressed via an unknown mechanism. MDS HSPCs and BMME cells are shown to have a bi-directional cross-talk between them. Healthy HSPCs in the BM of MDS patients are somehow reduced in numbers. Cells in the BMME such as MSCs and endothelial cells have been shown to be dysfunctional. Significant increase in the MDSCs and monocytes has been observed in MDS BM. Monocytes/Macrophages also seem to have changed in numbers and are dysfunctional. Abnormal levels of cytokines, growth factors and ROS is present in the MDS BMME that directly influence the MDS HSPCs.  $\oplus$  = Disrupted;  $\uparrow$  = Increase,  $\downarrow$  = Decrease. HSPCs, hematopoietic stem and progenitor cells; BMME, Bone marrow microenvironment; ROS, reactive oxygen species.

BM has been reported in AML xenograft mouse models, where changes in the perivascular niche, as a result of autocrine and paracrine secretion of vascular endothelial growth factor (VEGF) and other factors by leukemic cells, causes an increased proliferation of microvascular endothelium.<sup>35</sup> In another report, leukemic cells have been shown to push differentiation of MSCs towards an aberrant pro-fibrotic osteoblastic lineage, that in-turn promotes the expansion of leukemic cells at the expense of normal hematopoiesis.<sup>36</sup> It is therefore tempting to speculate that, like in leukemia, a combinatorial effect of ageing along with genetic abnormalities in MDS stem cells transmit 'disease cues' to the BMME that in-turn provides a nurturing niche for the sustenance of disease clones and at the same time, suppresses healthy HSPCs, and even contributes to the emergence as well as evolution of neoplastic clones.

Although there are some mouse models that have been developed to understand MDS pathophysiology, however their relevance as well as applicability to the disease remains debatable due to the complex nature of MDS patient clinical phenotypes. One of the first mouse models that provided experimental evidence specifically for the role of BMME, in this case an osteolineage cells, in the initiation of BM failure, was Dicer1 deleted murine model that generated myelodysplasia-like syndrome.<sup>37</sup> Transgenic mouse models (NUP98/ HOXD13) that develop MDS-like features, have reduced osteoclasts, and an increased in non-mineralized bones due to an increase in osteoblasts.<sup>38,39</sup> Increased level of fibroblast growth factor-23 (FGF-23) serum levels, that inhibits bone mineralization and erythropoiesis, were also observed in these mice. Interestingly, primary samples from MDS patients demonstrated similar levels.<sup>40</sup> In another mouse model that recapitulates pre-leukemic disorder Schwachman-Diamond syndrome (SDS), deletion of Sdbs gene in osteo-progenitors caused activation of the p53-S100A8/9-TLR axis with consequent oxidative genotoxicity in HSPCs and MDS-like transformation.<sup>41</sup>

## IS IMMUNO-NICHE A BYSTANDER?

Evolving hematopoiesis and associated immunological changes particularly in the innate immune compartment as well as cytokines/chemokines in the BM milieu are associated with the aging process, but are also particular hallmarks, of MDS (**Figure 1**). Over the years, studies have linked changes in the BMME and malignant hematopoiesis, however the mechanism leading to MDS-associated immune suppression is still largely unknown and in-fact remains controversial.<sup>24</sup> Elevated levels of cytokines, such as TNF, IFNs, TGF $\beta$ , IL-1, IL-6 and IL17, have been reported to play a supporting role in the maintenance of MDS disease.<sup>24,42-44</sup> Experimental evidence suggests that these cytokines can be produced not only by resident immune cells in the BM and circulating lymphocytes, but also by the stromal cells and HSPCs, however, the exact source remains unknown. The inflammatory molecules that cause constitutive activation of TLR-signaling and subsequent activation of mitogen-activated protein kinase (MAPK) as well as nuclear factor kappa B (NF- $\kappa$ B) have also been implicated in the pathogenesis of MDS.<sup>45-48</sup> Damage-associated molecular pattern (DAMP)-induced inflammation has been observed in the MDS BM. This is mediated via pyroptosis and can cause uncontrolled activation of inflammasome machinery eventually leading to HPSC lytic cell death and can act as a pathogenic driver of ineffective hematopoiesis in MDS. Oxidized mitochondrial DNA levels were reported to be higher in MDS than in the other overlapping syndromes and reactive conditions, such as CMML, CHIP, and anemia. Mesenchymal cells from MDS patients have been shown to induce genotoxic stress through p53-S100A8/9-TLR4 inflammatory signalling pathway, therefore leading to mitochondrial dysfunction, oxidative stress, and activation of DNA damage responses in HSPCs.<sup>41,49</sup> The phenomenon of aberrant activation of inflammasome machinery could at least partially elucidate the increased cell

death generally observed in the BM of MDS patients. Interestingly, induction of S100A9/S100A8 can result in p53-dependent erythroblasts differentiation defects, that is similar to del(5q) MDS clinical phenotype.<sup>50</sup>

Although the classical innate immune cell, namely monocytes that do not display any visible morphological changes, have been reported to be increased in number and this increase strongly associated with poor prognosis of MDS patients.<sup>51,52</sup> In fact, higher expression of thrombomodulin, a molecule with anti-inflammatory properties, is also reported in MDS derived monocytes.<sup>53</sup> It remains to be determined whether the changes in the output as well as the anti-inflammatory phenotype in the monocytes is driven directly by an intrinsic transcriptional machinery or via an intermediate environmental factor. For example, healthy donor derived monocytes when cocultured with MDS MSCs acquired phenotypic, metabolic as well as the functional properties of myeloid-derived suppressor cells (MDSCs), and eventually led to the suppression of NK cell function as well as proliferation of T-cells.<sup>54</sup> This BMME MSC driven mechanism indicates that suppression of the immune cells can be initiated from the malignant cells to its stroma via an indirect mechanism, therefore positively impacting the MDS clones. Furthermore, activation of monocytes in the BM by IL-36 impacts the survival and proliferation of leukemic cells, which ultimately inhibits the CD8+ T cell-mediated clearance of the leukemic blasts. Interestingly, IL-36 produced by leukemic progenitor cells facilitates their own proliferation, indirectly via activation of monocytes.<sup>55</sup> The existence of such self-reinforcing and protective mechanisms in leukemias, could explain some aspects of MDS-induced BMME remodeling, as a mechanism for disease progression and therapy failure.

Myeloid-derived suppressor cells (MDSCs) that are also part of the innate immune system and associated with immunosuppression in other cancers, are reported to be significantly increased in MDS

patients. MDSCs have been suggested to be distinct from the MDS neoplastic clone and associated with impaired hematopoiesis via a mechanism driven, at least partly by the interaction of S100A9 with an endogenous ligand for CD33-initiated signaling, which triggers the production of immunosuppressive cytokines IL-10 and TGF- $\beta$  to directly inhibit hematopoiesis.<sup>56-57</sup> Interestingly, MDS-like impaired hematopoiesis was reversed by MDSC depletion or inhibition of the S100A9/CD33/TLR4 axis.<sup>56</sup> Questions remain as to whether MDSCs are directly implicated or are being recruited/educated by cells in the BMME. Further studies are thus needed to fully understand their role in MDS.

## CONCLUSION

Research over the last decade has highlighted the importance of complex network of interactions, both physical as well as nonphysical, in homeostasis and during malignant state. Insights into the BMME has significantly advanced to recognize specific cell partners, modes of cell-cell communications, cellular differentiation trajectories and intrinsic as well as extrinsic biochemical pathways that form the fundamental basis of specific biological outcomes. Although it is well established that the sequential accumulation of genetic mutations in HSPCs is an essential event in the initiation of MDS, there is growing evidence implicating BMME in the maintenance as well as progression of disease and in response to treatment. There is an urgent need to understand the role of various 'specialized niches' in the BM where clonal HSPCs are residing, especially during the early stage of clonal development. Furthermore, deconvoluting the signaling landscape that MDS HSPCs use to either remodel their environment as well as suppress their normal cohabitating HSPCs will be of great therapeutic potential. Certainly, gaining the understanding of how intrinsic and extrinsic inflammation factors contribute to HSPC aging and development of MDS, in general, might also provide novel therapeutic options that can be potentially used alongside existing treatments.

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# MEETING HIGHLIGHTS AND ANNOUNCEMENTS

THE 64TH AMERICAN SOCIETY OF HEMATOLOGY ANNUAL MEETING AND EXPOSITION 2022 (ASH 2022)

## BREAKFAST SYMPOSIUM

DECEMBER 9, 2022

# MYELODYSPLASTIC SYNDROMES 2022: TODAY AND TOMORROW

NEW ORLEANS, LOUISIANA

LANOUELLE ORLEANS BALLROOM AB • NEW ORLEANS ERNEST N. MORIAL CONVENTION CENTER  
FRIDAY, DECEMBER 9, 2022, 7:00 AM–10:00 AM CT

### ACTIVITY OVERVIEW

The 2022 Symposium will focus on recent advances in the diagnosis, classification and management of patients with myelodysplastic syndromes (MDS). The program combines evidence on current practice with information that might be applied in the future.

### AGENDA TOPICS

- The History and Future of MDS
- When are erythroid stimulating agents (ESA) effective and what can we offer after their failure?
- Real-world data and clinical trials – does it matter?
- Inflammaging, comorbidities and VEXAS syndrome in MDS pathogenesis
- Molecular prognostic scoring (IPSS-M) – How does it improve patient management?
- Anemia: Effects on quality of life and organ function
- Patient discussion session

### TARGET AUDIENCE

This activity is designed for an audience of pharmacists, physicians, and nurses.

### LEARNING OBJECTIVES

**Participants will learn about novel information obtained over the last decade and will hear about potential applications in the field of MDS.**

- Review the history of myelodysplastic syndromes.
- Project future developments of myelodysplastic syndromes.

- Analyze current evidence of the use of ESAs in MDS and the potential alternatives as a second line treatment.
- Compare the contribution of real-world data with evidence obtained in clinical trials explaining how to address this information.
- Recognize the role that inflammation has in MDS pathogenesis and the influence of aging and comorbidities on these diseases.
- Identify the new molecular classification (IPSS-M) of MDS as well as the role of genetic testing in patient evaluation.
- Describe the influence of anemia on quality of life and other non-hematopoietic organ function.
- Discuss management of patients with MDS

### FACULTY

**Moshe Mittelman, MD** –  
Symposium Co-Chair

**Valeria Santini, MD** – Symposium Co-Chair

**Stephen Nimer, MD** – MDSF Chairman

**Rafael Bejar, MD, PhD**

**Rena Buckstein, MD**

**Jane Churpek, MD**

**Theo M. de Witte, MD, PhD**

**Peter Grayson, MD, Msc**

**Elizabeth A. Griffiths, MD**

**Yasushi Miyazaki, MD, PhD**

**Sophie Park, MD, PhD**

**Lewis Silverman, MD**

### AGENDA

**7:00 – 7:10 am**

Welcome

**7:05 – 7:15 am**

*The History and Future of MDS  
Program Overview and Objectives*

**7:15 – 7:35 am**

*When are erythroid stimulating agents (ESA) effective and what can we offer after their failure?*

**7:35 – 7:55 am**

*Real-world data and clinical trials – does it matter?*

**7:55 – 8:15 am**

*Inflammaging, comorbidities and VEXAS syndrome in MDS pathogenesis*

**8:15 – 8:30 am**

*Molecular prognostic scoring (IPSS-M) – How does it improve patient management?*

**8:30 – 8:55 am**

*Are we ready to perform NGS in all MDS patients?*

**8:55 – 9:15 am**

*Anemia and Transfusion Dependence: Effects on quality of life and organ function*

**9:15 – 9:55 am**

*Patient Discussion Session – Including two MDS patient case studies*

*Expert Panel*

**9:55 – 10:00 am**

*Closing Remarks*

**PLEASE MAKE SURE TO VISIT THE MDS FOUNDATION BOOTH #2522 IN THE EXHIBIT HALL!**

# WELCOME TO MDS 2023

On behalf of the Local Organizing Committee and the MDS Foundation, it is our pleasure to invite you to **the 17<sup>th</sup> International Congress on Myelodysplastic Syndromes taking place in Marseille, France on May 3-6, 2023.**

For more than 20 years, every two years, the MDS International Meeting has been a milestone for those who are interested in myelodysplastic syndromes.

During three days at MDS 2023, renowned international experts will present and discuss the recent advances in the field. The congress will cover basic and translational research, diagnosis, prognosis, new therapies and patient experiences. It will include several workshops and round tables in order to foster discussion. High-level research results, selected from the abstracts submitted by colleagues will also be presented.

Due to the COVID 19 pandemics, the previous meeting was held virtually. In May 2023, you are all expected in Marseille, where you can enjoy its 2600-year-old cultural heritage, its amazing natural site on the shores of the Mediterranean Sea, its friendliness and great food.

We hope we will rediscover the taste of being together to share knowledge and experience in a lovely place and friendly environment.

We look forward to seeing you in Marseille!

**Norbert Vey and Pierre Fenaux**  
Congress Co-Chairs

**ABSTRACT SUBMISSION DEADLINE:**  
**17 JANUARY 2023**

**EARLY REGISTRATION DEADLINE:**  
**7 MARCH 2023**



# MARSEILLE



THE 17<sup>TH</sup> INTERNATIONAL CONGRESS ON  
**MYELODYSPLASTIC SYNDROMES**

Marseille, France | 3-6 May 2023



**LEGEND:**

Plenary Session	Workshop	Meet the Expert	Young Investigators Award	Nurses Program	Networking Event	Industry Session
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**WEDNESDAY, MAY 3, 2023**

	HALL A	HALL B	HALL C	HALL D
14:30-15:30	Nurses Workshop: The Nurse and the MDS Patient Management	Workshop I: Academic Clinical Trials in MDS /CMML	Workshop II: New Classifications of MDS/CMML, and Response Criteria	
15:30-15:45	Break			
15:45-16:45	Nurses Workshop Continues	Workshop I Continues	Workshop II Continues	
16:45-17:00	Break			
17:00-18:00	Industry Supported Session Not Included in main event CME/CPD Credit			
18:00-18:15	Break			
18:15-19:45	Opening Ceremony & Keynote Lecture			
19:45	Welcome Reception (Exhibition Area)			

**THE 17<sup>TH</sup> INTERNATIONAL CONGRESS ON  
MYELODYSPLASTIC SYNDROMES**

Marseille, France | 3-6 May 2023



**LEGEND:**

Plenary Session	Workshop	Meet the Expert	Young Investigators Award	Nurses Program	Networking Event	Industry Session
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**THURSDAY, MAY 4, 2023**

	HALL A	HALL B	HALL C	HALL D
08:00-08:45	(07:45-08:45) <b>Industry Supported Session II</b> Not Included in main event CME/CPD Credit	<b>Meet the Expert I:</b> How to Treat Higher Risk MDS After HMA Failure	<b>Meet the Expert II:</b> What can Medical Intelligence Currently Bring to the Management of MDS?	<b>Meet the Expert III:</b> Treatment of Anemia of Lower Risk MDS After ESA and Lenalidomide Failure
08:45-09:00	Break			
09:00-10:30	<b>Plenary Session I:</b> Dysimmunity, Inflammation and MDS			
10:30-11:00	Coffee Break, Poster Viewing & Visit the Exhibition			
11:00-12:30	<b>Plenary Session II:</b> From Clonal Hematopoiesis to Overt MDS			
12:30-14:00	(12:45-13:45) <b>Industry Supported Session III - Pipeline session</b> Not Included in main event CME/CPD Credit	Lunch Break, Poster Viewing & Visit the Exhibition		
14:00-15:30	<b>Plenary Session III:</b> Prognostication and Stratification of MDS			
15:30-16:00	Coffee Break, Poster Viewing & Visit the Exhibition			
16:00-17:30	<b>Plenary Session IV:</b> Dyserythropoiesis in MDS			
18:00-19:00	Poster Viewing & Visit the Exhibition			

# THE 17<sup>TH</sup> INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES

Marseille, France | 3-6 May 2023



## LEGEND:

<b>Plenary Session</b>	<b>Workshop</b>	<b>Meet the Expert</b>	<b>Young Investigators Award</b>	<b>Nurses Program</b>	<b>Networking Event</b>	<b>Industry Session</b>
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## FRIDAY, MAY 5, 2023

	HALL A	HALL B	HALL C	HALL D
08:00-08:45	(07:45-08:45) <b>Industry Supported Session IV</b>  Not Included in main event CME/CPD Credit	<b>Meet the Expert IV:</b> The New Molecular IPSS in Clinical Practice	<b>Meet the Expert V:</b> When and How to Perform AlloSCT in MDS and CMML	<b>Meet the Expert VI:</b> Issues in the Classification of MDS and in Response Criteria: The Devil is in the Details
08:45-09:00	Break			
09:00-10:45	<b>Plenary Session V:</b> Predisposition to MDS			
10:45-11:05	Coffee Break, Poster Viewing & Visit the Exhibition			
11:05-12:35	<b>Plenary Session VI:</b> Druggable Mutations and Other Personalized Approaches for the Treatment of MDS			
12:35-14:00	(12:45-13:45) <b>Industry Supported Session V</b>  Not Included in main event CME/CPD Credit	Lunch Break, Poster Viewing & Visit the Exhibition		
14:00-15:30	<b>Plenary Session VII:</b> Non-Targeted Approaches for HR MDS			
15:30-16:00	Coffee Break			
16:00-17:30	<b>Plenary Session VIII:</b> Outcome Researches			
17:30-18:30	Poster Viewing & Visit the Exhibition			
19:30	Networking event			

**THE 17<sup>TH</sup> INTERNATIONAL CONGRESS ON  
MYELODYSPLASTIC SYNDROMES**

Marseille, France | 3-6 May 2023



**LEGEND:**

<b>Plenary Session</b>	<b>Workshop</b>	<b>Meet the Expert</b>	<b>Young Investigators Award</b>	<b>Nurses Program</b>	<b>Networking Event</b>	<b>Industry Session</b>
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**SATURDAY, MAY 6, 2023**

	HALL A	HALL B	HALL C	HALL D
08:00-09:45	<b>Plenary Session IX:</b> CMML and Other Specific Sub-Types of MDS and MDS/MPN			
09:45-10:15	Coffee Break, Poster Viewing & Visit the Exhibition			
10:15-11:45	<b>Plenary Session X:</b> Innovations in Transplantation and Cellular Immunotherapy for MDS Patients			
11:45-12:15	<b>Keynote Lecture: ICC &amp; WHO Classification debate</b>			
12:15-13:15	<b>The Tito Bastianello and MDSF Young Investigators Awards</b>			
13:15-13:30	<b>Closing Ceremony</b>			



## REGISTRATION

Registration fees EUR apply to payments received prior to the indicated deadlines.

	EARLY RATE Until Mar 7, 2023	REGULAR RATE Until April 19, 2023	ONSITE RATE From April 20, 2023
<b>MDSF Member</b>	<b>675 EUR</b>	<b>795 EUR</b>	<b>895 EUR</b>
<b>Non Members</b>	<b>795 EUR</b>	<b>895 EUR</b>	<b>990 EUR</b>
<b>Clinical Care/Allied Health</b> (i.e. nurses, pharmacists, physician assistants)	<b>225 EUR</b>	<b>275 EUR</b>	<b>325 EUR</b>
<b>Student/Hematologists in Training</b>	<b>200 EUR</b>	<b>225 EUR</b>	<b>275 EUR</b>
<b>Morphology Workshops</b>	<b>50 EUR</b>	<b>50 EUR</b>	<b>80 EUR</b>
<b>Networking Dinner</b>	<b>75 EUR</b>	<b>75 EUR</b>	<b>75 EUR</b>

- \* MDS Foundation members benefit from reduced fees. You can now become a member during registration by visiting the [MDSF Membership website](#).
- \*\* In order to benefit from the special fee, a submission of your status confirmation (approval letter signed by the Head of Department or copy of your status ID) must be uploaded during online registration.
- \*\*\* The Workshops have limited availability and registration is on a first-come first-serve basis.

### FEES FOR ALL MEETING PARTICIPANTS INCLUDE

- Participation in all scientific sessions
- Entrance to the Exhibition
- Invitation to the Opening Ceremony & the Welcome Reception
- Coffee & lunch during breaks, as indicated in the program
- Printed material of the Symposium
- Certificate of attendance

### PAYMENT METHODS

Payment of registration fees must be made in EUR after completing the registration process. You may choose one of the following methods:

**Credit Card:** i.e. Visa, MasterCard or American Express

**Bank Transfer:** Additional 30 EUR handling fee is required.

Please make drafts payable to:

#### MDS 2023 Congress, Marseille

Credit Suisse Bank, Geneva Branch, 1211 Geneva 70, Switzerland

Clearing number: 4835

Account number: 1500934-92-270

Swift code: CRESCHZZ80A

IBAN number: CH97 0483 5150 0934 9227 0

- Please ensure that the name of the conference and participant are stated on the bank transfer.
- Bank charges are the responsibility of the participant and should be paid at source in addition to the registration fees.
- Registration will only be valid upon receipt of the full payment by the registration department according to the deadline indicated. An email confirming registration will only be sent after receipt of the required fees.

- Outstanding payments will be collected on-site and charged the on-site rate. A copy of the bank transfer (or other proof of payment) will be required in the event that registration fees were not credited to the Conference account on time.

### CANCELLATION POLICY

All cancellations must be emailed to [reg\\_mds23@kenes.com](mailto:reg_mds23@kenes.com) prior to the below deadlines. Refund of the registration fee will be as follows:

- Cancellations received up to and including March 8, 2023 – full refund
- Cancellations received from March 9 until April 19, 2023 – 50% refund
- From April 20, 2023 – no refund will be made

Note, in case of cancellation at any stage, the Bank Transfer handling fee (30 EUR) will not be refunded – applicable to Bank Transfer payments only.

### GROUP REGISTRATION

For group registration of 10 delegates or more, companies are requested to contact the MDS Registration Team at: [reg\\_mds23@kenes.com](mailto:reg_mds23@kenes.com)

### GUEST ATTENDANCE POLICY

All event activities (including educational sessions, meal functions, exhibition hall, etc.) are exclusively reserved for registered attendees. Non-registered guests (including children, family members, colleagues, etc.) are not allowed in any of the event areas. Badges provided at registration are required for entrance into all functions and will be strictly enforced.

# INTERNATIONAL WORKING GROUP

## MDS FOUNDATION INTERNATIONAL WORKING GROUP FOR PROGNOSIS IN MDS

### MOLECULAR INTERNATIONAL PROGNOSTIC SCORING SYSTEM DEVELOPED FOR MDS

**YARDVILLE, N.J. JUNE 12, 2022 – (BUSINESS WIRE).** Today, the MDS Foundation announced the development of a new prognostic scoring system, the IPSS-Molecular, that will significantly improve risk stratification upon diagnosis to better inform the way treatment plans are built for patients with Myelodysplastic Syndromes.

“By considering each patient’s genetic makeup in the IPSS-M we can now truly deliver patient tailored risk stratification,” says Dr. Elli Papaemmanuil of the Memorial Sloan Kettering Cancer Center. “This will enable optimized treatment decisions tailored to those most in need of intervention and sets a biological framework for the design of future clinical trials.”

For more than two decades, MDS patient risk stratification at diagnosis was based on diagnostic blood counts, morphology, and cytogenetics through the International Prognostic Scoring System. In recent years, a complete catalog of the genes mutated in MDS has been discovered. However, even though patients increasingly receive panel gene testing at diagnosis, there were no guidelines as to how this information could be used clinically to guide risk stratification and treatment decisions.

The creation of an effective set of prognostic guidelines was especially important for MDS because current clinical courses are very heterogeneous. In this absence of targeted therapies, risk stratification at diagnosis is critical in guiding treatment decisions. For example, supportive care with less toxic treatments would be considered for low-risk patients, while patients with high-risk disease need higher intensity treatments to mitigate the risk of leukemia progression and death.

### AN INTERNATIONAL COLLABORATION TO IMPROVE RISK STRATIFICATION

Through the efforts of the International Working Group for the Prognosis of MDS—a consortium of expert clinicians, pathologists, computational biologists, and statisticians—and under the auspices of the MDS Foundation, a total of 2,957 patient blood and marrow samples were collected from 24 centers within 13 countries. Those samples were all sequenced

uniformly by a panel test at Memorial Sloan Kettering Cancer Center and analyzed in the Papaemmanuil laboratory. Each sample was profiled for mutations in 152 genes and clinical and molecular variables were evaluated for associations with leukemia-free survival, leukemic transformation, and overall survival. In order to validate the IPSS-M, the study used another 754 samples derived from the Japanese MDS consortium. The results of this investigation, led by Drs Elsa Bernard, Elli Papaemmanuil and IWG colleagues, ‘Molecular International Prognostic Scoring System for MDS (IPSS-M)’, have just been published in the June 12, 2022, issue of the *New England Journal of Medicine Evidence*.

### DETERMINING THE IPSS-M RISK SCORE AND RISK CATEGORIES

The study found at least one driver genomic alteration in 94% of patients (41% of patients with cytogenetic alterations and 90% of patients with gene mutations). On average, the higher the number of alterations, the worse the outcomes.

Using this data, researchers developed the IPSS-Molecular which incorporates information from 31 gene mutations as well as diagnostic and clinical parameters to deliver a personalized, patient-specific risk score. The IPSS-M risk score indicates the continuum of prognostic risk observed across patients with MDS and produces six risk categories (Very Low, Low, Moderate Low, Moderate High, High, Very High) that can be used for determining clinical trial eligibility criteria, correlative studies, and treatment recommendations.

In addition to its effective prognostic categorization of primary MDS, the IPSS-M efficiently stratifies patients with therapy-related MDS, a diagnostic subtype that was considered to be uniformly high risk and therefore lacked an applicable prognostic system. Compared to the previous IPSS-R system, 46% (roughly 1 in 2) of patients were reassigned to a different risk category in the IPSS-M which led to improved prognostic discrimination. Importantly, 7% of patients were reassigned by more than one strata (e.g IPSS-R Low to IPSS-M High or vice versa).

### THE FUTURE OF MDS RISK STRATIFICATION

The IPSS-Molecular is set to become a new international standard for risk stratification of MDS patients at diagnosis. The IPSS-M score is

personalized, interpretable, reproducible, and provides a flexible and transparent strategy to account for missing values. It also provides a list of the 31 genes that must be considered in diagnostic assay design.

To support widespread adoption and use, the research team developed a web-based calculator where clinicians can enter patient clinical and molecular data to deliver a personalized prediction of risk, outcomes, and likelihood of leukemia transformation in order to guide cost-benefit analysis on treatment decisions.

“It has been a privilege to work closely with expert MDS clinicians, pathologists, biologists, statisticians, and the broader IWG community to develop the IPSS-M, aiming to better guide and ultimately improve the care of patients with MDS,” says Dr. Elsa Bernard of the Memorial Sloan Kettering Cancer Center. “While the IPSS-M can be calculated using a relatively simple formula, we built a web calculator and an R package to facilitate its application, which we hope will prove useful for the community.”

### EXPLORE THE WEB-BASED

**IPSS-M CALCULATOR:** [https://](https://www.mds-foundation.org/mds-iwg-pm/)

[www.mds-foundation.org/mds-iwg-pm/](https://www.mds-foundation.org/mds-iwg-pm/)

**READ THE IPSS-M STUDY:** [https://](https://evidence.nejm.org/doi/full/10.1056/EVIDo)

[evidence.nejm.org/doi/full/10.1056/EVIDo](https://evidence.nejm.org/doi/full/10.1056/EVIDo)  
a2200008

### STUDY INVESTIGATORS:

Elsa Bernard, PhD, Heinz Tuechler, Peter L. Greenberg, MD, Robert P. Hasserjian, MD, Juan E. Arango Ossa, M.S., Yasuhito Nannya, MD, PhD, Sean M. Devlin, PhD, Maria Creignou, MD, Philippe Pinel, M.S., Lily Monnier, M.S., Gunes Gundem, PhD, Juan S. Medina-Martinez, M.S., Dylan Domenico, B.S., Martin Jädersten, MD, PhD, Ulrich Germing, MD, Guillermo Sanz, MD, PhD, Arjan A. van de Loosdrecht, MD, PhD, Olivier Kosmider, MD, PhD, Matilde Y. Follo, PhD, Felicitas Thol, MD, Lurdes Zamora, PhD, Ronald F. Pinheiro, PhD, Andrea Pellagatti, PhD, Harold K. Elias, MD, Detlef Haase, MD, PhD, Christina Ganster, PhD, Lionel Ades, MD, PhD, Magnus Tobinsson, MD, PhD, Laura Palomo, PhD, Matteo Giovanni Della Porta, MD, Akifumi Takaori-Kondo, MD, PhD, Takayuki Ishikawa, MD, PhD, Shigeru Chiba, MD, PhD, Senji Kasahara, MD, PhD, Yasushi Miyazaki, MD, PhD, Agnes Viale, PhD, Kety Huberman, B.S., Pierre Fenaux, MD, PhD, Monika Belickova, PhD, Michael R. Savona, MD, Virginia M. Klimek, MD, Fabio P. S. Santos, MD, PhD, Jacqueline Boulwood, PhD, Ioannis Kotsianidis, MD, PhD, Valeria Santini, MD, Francesc Sole, PhD, Uwe Platzbecker, MD, Michael Heuser, MD, Peter Valent, MD, Kazuma Ohyashiki, MD, PhD, Carlo Finelli, MD, Maria Teresa Voso, MD, Lee-Yung Shih, M.S., Michaela Fontenay, MD, PhD, Joop H. Jansen, PhD, Jose Cervera, MD, PhD, Norbert Gattermann, MD, Benjamin L. Ebert, MD, PhD, Rafael Bejar, MD, PhD, Luca Malcovati, MD, Mario Cazzola, MD, Seishi Ogawa, MD, PhD, Eva Hellstrom-Lindberg, MD, PhD, and Elli Papaemmanuil, PhD.

## MDS RISK ASSESSMENT CALCULATORS

THE IWG-PM UNDER THE AEGIS OF THE MDS FOUNDATION, INC. HAS DEVELOPED TWO PROGNOSTIC TOOLS, **THE IPSS-M AND IPSS-R CALCULATORS**, TO DETERMINE A PATIENT'S RISK OF PROGRESSING TO ACUTE MYELOID LEUKEMIA (AML).

### NEW IPSS-M CALCULATOR

The IPSS-M is the newest MDS prognosis calculator that combines genomic profiling with hematologic and cytogenetic parameters, improving the risk stratification of patients with MDS. This is a valuable tool for clinical decision-making, offering the prospect of tailoring diagnosis and therapeutic interventions to each patient's molecular profile. *iOS and Android apps coming soon.*

<https://www.mds-foundation.org/mds-iwg-pm/>

### IPSS-R CALCULATOR

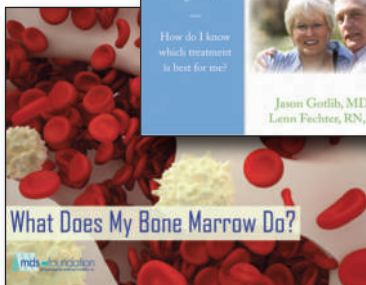
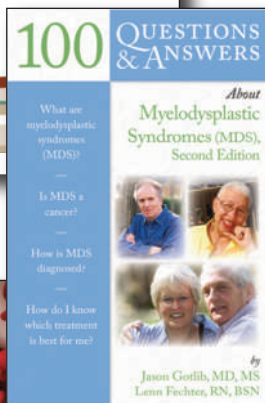
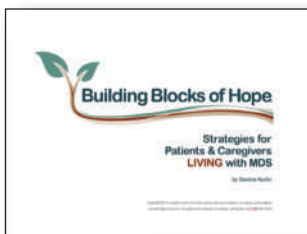
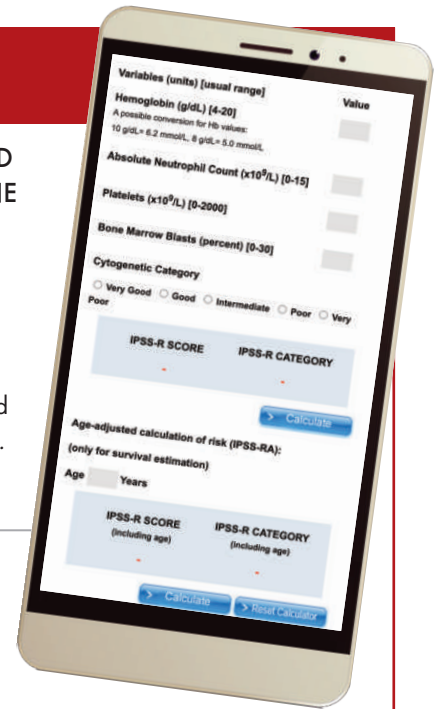
The IPSS-R is the current MDS prognosis calculator that combines hematologic and cytogenetic parameters to determine an MDS patient's risk stratification. This calculator tool includes clinical features of marrow blasts, cytogenetics, depth of cytopenias and age as well as the additive differentiative features for patient survival of performance status, serum ferritin, LDH, beta-2 micro globulin and marrow fibrosis.

<https://www.mds-foundation.org/advanced-calculator>

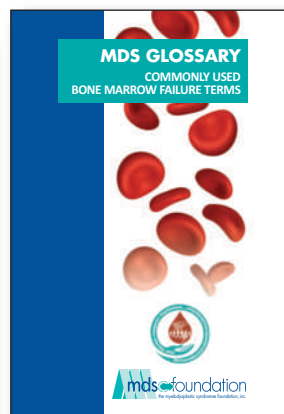


**DOWNLOAD IPSS-R CALCULATOR APP**

<https://play.google.com/store/apps/details?id=com.basiccalculator>



## FIND THE TRUSTED RESOURCES YOU NEED... YOU OR SOMEONE YOU KNOW HAS BEEN DIAGNOSED WITH MDS



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

Dealing with MDS can be very difficult, but it helps to have resources that are reliable and easy to understand.

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

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

## INTERNATIONAL WORKSHOP ON MDS MEETING REPORT

The inaugural *International Workshop on Myelodysplastic Syndromes (iwMDS)* took place on 24–26 June 2022 in Miami, FL. Following the success of the iwAL meetings established since 2018, which focused on acute leukemias, *The Video Journal of Hematological Oncology (VJHemOnc)* and leading clinicians within the MDS field came together to organize a workshop dedicated to addressing the key issues in treating patients with MDS, and to shine a spotlight on the advances in clinical research. The 1st iwMDS meeting brought together over 50 faculty members and allowed many of them a chance to meet again in person after the pandemic to network with each other and debate current issues, particularly in a setting more intimate and cohesive for peer-to-peer discussion compared with large congresses.

The presentations and roundtable discussions were published for on-demand viewing on VJHemOnc.com post-event and have received over 2,500 views to date, highlighting the relevance of information discussed well after the event.

VJHemOnc is an independent, open-access video journal that provides healthcare professionals across the globe with the latest research and educational updates in hematological oncologies from key scientific congresses throughout the year. The MDS Foundation and VJHemOnc have partnered to help deliver educational events for both patients and HCPs. The iwMDS meeting was pleased to have the MDS Foundation participate, represented by Tracey Iraca, Executive Director of the MDS Foundation.

On the Friday, the packed agenda kicked off with a Fellow & Junior Faculty Career



Development session dedicated to the various career development opportunities available, including the MDS Foundation's grant, networking and mentoring opportunities. The second session delved into Morphologic classification of MDS and defining the boundary between MDS & other neoplasms. Both sessions were streamed live to a virtual audience of over 300 total viewers.

Saturday & Sunday's session topics included Improving the efficiency of drug approval, Inflammation, immune dysregulation and targeting, Updates in classification and risk stratification, Clonal hematopoiesis and Pre-MDS states, Splicing factor and RNA pathobiology/epigenetics, and Non-interventional & Big Data Research.

Positive feedback from the faculty members highlighted the success of the 1st iwMDS

meeting — including a net promoter score (NPS) of 87 and rating of 4.6/5 on the content covered and production of the meeting.

A special thanks goes to Amer Zeidan, iwMDS Chair, and the iwMDS Scientific Committee members Omar Abdel-Wahab, Rena Buckstein, Valeria Santini, Michael Savona and Andrew Wei, for putting together an excellent agenda and bringing together leading MDS clinicians in order to help improve outcomes for patients with MDS.

Another note of gratitude goes to Gilead, Novartis, Bristol Myers Squibb, Syros, Geron and Karyopharm for supporting the 1st iwMDS meeting.

Planning of the 2nd iwMDS meeting in 2023 is underway — to get in touch for any further information, please visit:

[www.iwMDS.org/contact](http://www.iwMDS.org/contact).





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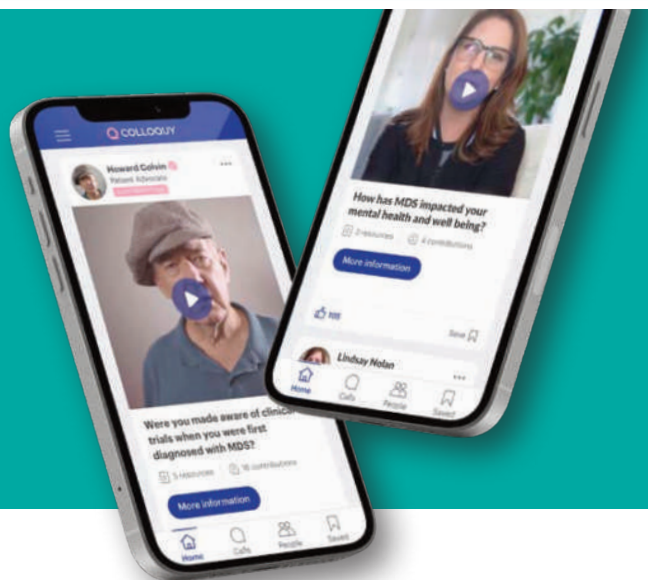
Victoria, Australia

## FROM THE MDS FOUNDATION

# Share In Confidence

Colloquy provides you with a safe and supportive environment to share your personal stories and hear real-life experiences from patients and carers like you.




**WE HAVE LAUNCHED!**



Colloquy in MDS is brought to you in partnership with the MDS Foundation.



### FOLLOW THREE EASY STEPS:

-  **Step 1**  
Sign up to Colloquy: [mds.colloquy.health/](https://mds.colloquy.health/)
-  **Step 2**  
Listen to and share experiences with others.
-  **Step 3**  
Understand more about your MDS from patients and carers like you.

### How can you help others?

Your experiences will help the MDS Foundation uncover the true unmet needs associated with your condition, informing future patient support programs and research.



**Sign up today!**  
[mds.colloquy.health/](https://mds.colloquy.health/)





# MEET JOE!



**JOE** represents a nucleus, bringing everything (information and resources) together in one place. JOE takes patients, caregivers and loved ones on a Journey Of Empowerment, **allowing them to be their own best advocates**



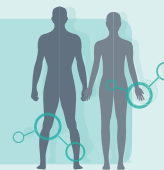
### Module 1

The essential facts of MDS



### Module 2

Understanding the impact of MDS on your body



### Module 3

Understanding your MDS diagnosis



### Module 4

Managing the signs and symptoms of MDS



### Module 5

Treatment of MDS



### Module 6

Patient empowerment



## LAUNCHING LATER THIS YEAR!



**JOE** will be available online, through any web browser or mobile application



*MDS JOE is brought to you by the MDS Foundation*

**QUESTIONS  
ANSWERED**



**VISIT OUR  
WEBSITE**



**DO YOU KNOW YOUR MDS SUBTYPE, IPSS-R SCORE & GENE MUTATION PROFILE?**

MDS treatment is individualized based on a patient’s subtype, IPSS-R score and, to some extent, genetic mutation. This knowledge will empower patients and their caregivers to take a more active role in decisions about their treatment and advocate for appropriate treatments that may prolong their life and improve their quality of life. The following information is designed to help you understand how your subtype and IPSS-R score are determined, as well as general information on genetic mutations commonly found in MDS and the importance of genetic testing for these mutations. Knowing your subtype, IPSS-R score and gene mutation profile will help facilitate discussions with your healthcare provider on what this means for you personally and help select the best treatment options.

**IPSS-R SCORE**

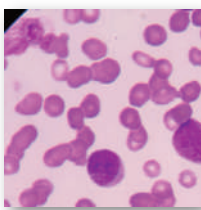
The IPSS-R is a classification system used by doctors to help predict a person’s risk of developing AML and overall survival without treatment.

**MDS SUBTYPE**

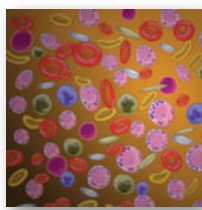
MDS is classified into several different subtypes based on the following features: Blood cell counts, Percentage of blasts in the bone marrow, and Cytogenetics.

**MUTATION PROFILE**

Genetic mutations occur when a gene is damaged and alters the genetic message. Mutations can potentially identify effective therapies to treat your disease.



**BONE MARROW  
BLAST**



**MDS-RS-MLD**



**CYTOGENETICS**



**UNDERSTANDING YOUR MDS: KNOW YOUR SCORE, YOUR SUBTYPE, AND YOUR MUTATION**

*This brochure is intended to help you better understand the diagnosis of MDS. Created by the MDS Foundation staff, Board of Directors, and medical and scientific leaders, it will explain the various MDS subtypes; how a prognostic scoring system is designed and where you can place yourself with the help of your physician and other health professionals. You will learn about normal and abnormal blood cells; leukemic blasts; blood counts; chromosomes and molecular mutations that may assist your provider in further modifying your subtype and, possibly, selecting the type of therapy for you.*

**John M. Bennett, MD**

First Chair and Founding Member of the MDS Foundation

To order your free copy of **UNDERSTANDING YOUR MDS: Know your Score, your Subtype, and your Mutation**, please call 1-609-298-1035 or order online at <https://www.mdsknowledgeispower.com/order-a-brochure/>.

**To learn more, visit our website at <https://www.mdsknowledgeispower.com/>.**

# Move FOR MDS

Community Walks to Drive Awareness & Accelerate Research

WE HAD AN AMAZING TIME RAISING FUNDS AND AWARENESS AT OUR 5 MOVE FOR MDS 5K AWARENESS WALKS. YOUR SUPPORT MEANS SO MUCH TO EVERYONE AFFECTED BY MYELODYSPLASTIC SYNDROMES.

**THANK YOU AGAIN FOR YOUR SUPPORT!  
WE CAN'T FIGHT MDS WITHOUT YOU.**

**LOS ANGELES**  
AUGUST 28

**CHICAGO**  
SEPTEMBER 25

**NYC**  
OCTOBER 2

**NASHVILLE**  
OCTOBER 15

**BOSTON**  
OCTOBER 23

**GLOBAL**  
OCTOBER 23



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SYROS | TAIHO ONCOLOGY | TENNESSEE ONCOLOGY | WINTRUST

# MDS CENTERS OF EXCELLENCE

Our MDS Centers of Excellence are institutions that meet the highest standards for diagnosis, treatment and patient care. These centers help patients seeking first or second opinions and/or additional treatment options from experts in MDS. We currently have 78 Centers in the United States and 121 Centers in countries around the world. Our MDS Centers can be viewed here:

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



## BENEFITS OF MEMBERSHIP:

- MDSF CoEs form the referral base for the patients who contact the Foundation daily.
- MDSF CoEs are proudly recognized on the Foundation website, within our printed newsletters, and through our various social media platforms.
- MDSF CoEs are offered discounted registration rates at MDS Foundation meetings and a 60% annual subscription discount to Leukemia Research.
- MDSF CoEs have full access to MDSF educational resources for distribution to your patients.
- In addition, along with your \$500 CoE renewal payment, your annual MDSF Professional Membership dues are waived. MDSF Professional Members are also listed, by name, on our website and in our printed newsletters.
- The work of your institution can be shared with our patient and professional contacts via our website and/or our social media channels. We can spread the word of your clinical trials, research projects, etc.

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

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

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

Please contact the Foundation for further information and an application form for your center. *The following centers have qualified as MDS Centers of Excellence:*

## UNITED STATES

### ALABAMA

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

### ARIZONA

Mayo Clinic Hospital  
Phoenix, Arizona  
Cecilia Arana Yi, MD

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 Koefler, MD

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

Moore's Cancer Center–UC San Diego Health  
San Diego, California  
Rafael Bejar, MD, PhD/Tiffany N. Tanaka, MD

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

UCLA Health Hematologic Malignancies and Stem Cell Transplant Program  
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  
Maria Amaya, MD, PhD – Practice Location:  
Rocky Mountain Regional VA  
Christine McMahan, MD – Practice Location:  
UCHealth Blood Disorders and Cell Therapies Center  
– Anschutz Medical Campus

### CONNECTICUT

Yale Cancer Center/Smilow Cancer Hospital  
Yale University School of Medicine  
New Haven, Connecticut  
Amer Zeidan, MD

### FLORIDA

Blood and Marrow Transplant Center  
AdventHealth Cancer Institute  
Orlando, Florida  
Juan Carlos Varela, MD, PhD

Mayo Clinic  
Jacksonville, Florida  
James M. Foran, MD

Moffitt Cancer Center  
Tampa, Florida  
Rami Komrokji, MD  
Alison R. Walker, MD

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

University of Florida Shands Hospital  
Gainesville, Florida  
Zeina Al-Mansour, MD

### GEORGIA

Emory Winship Cancer Institute  
Emory University School of Medicine  
Atlanta, Georgia  
Amelia Langston, MD  
Nikolaos Papadantonakis, MD, PhD, MSc

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

### ILLINOIS

Loyola University Chicago  
Cardinal Bernardin Cancer Center  
Maywood, Illinois  
Stephanie B. Tsai, MD

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

Rush University Medical Center  
Chicago, Illinois  
Melissa L. Larson, MD

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

## INDIANA

Indiana University Simon Cancer Center  
Indianapolis, Indiana  
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## IOWA

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and Clinics, Holden Cancer Center  
Iowa City, Iowa  
*Grerk Sutamtewagul, MD*

## KANSAS

The University of Kansas Cancer Center  
Westwood, Kansas  
*Barry Skikne, MD*

## 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

Dana-Farber/Boston Children's Cancer  
and Blood Disorders Center  
Boston, Massachusetts  
*Akiko Shimamura, MD, PhD*  
Dana-Farber Cancer Institute  
Boston, Massachusetts  
*Richard M. Stone, MD*  
*Benjamin Ebert, MD, PhD*  
Massachusetts General Hospital  
Cancer Center  
Boston, Massachusetts  
*Timothy Graubert, MD*  
Tufts Medical Center  
Boston, Massachusetts  
*Andreas Klein, MD*

## MICHIGAN

Barbara Ann Karmanos Cancer Institute  
Wayne State University  
Detroit, Michigan  
*Jay Yang, MD*  
William Beaumont Hospital Cancer Center  
Royal Oak, Michigan  
*Ishmael Jaiyesimi, DO*

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Rochester, Minnesota  
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Mrinal S. Patnaik, MBBS*  
University of Minnesota  
Medical Center, Fairview University  
of Minnesota Medical School  
Minneapolis, Minnesota  
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St. Louis, Missouri  
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Hackensack, New Jersey  
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Rutgers Cancer Institute of New Jersey  
Rutgers University Hematologic Malignancies  
and Stem Cell Transplant  
New Brunswick, New Jersey  
*Dale G. Schaar, MD, PhD*

## NEW MEXICO

University of New Mexico  
Comprehensive Cancer Center  
Albuquerque, New Mexico  
*Leslie Andritsos, MD/Ala Ebaid, MD*

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New York, New York  
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Texas Oncology – Austin Midtown  
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*Hagop Kantarjian, MD*

University of Texas, Southwestern Medical Center  
Dallas, Texas  
*Robert H. Collins, Jr., MD, FACP/Yazan Madanat, MD*

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Salt Lake City, Utah  
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University of Washington  
Seattle Cancer Care Alliance  
Seattle, Washington  
*Joachim Deeg, MD/Bart L. Scott, MD*

## WASHINGTON, DC

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*Catherine Broome, MD*  
George Washington University, VA Medical Center  
Washington, D.C.  
*Anita Aggarwal, DO*

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University of Wisconsin Health  
Madison, Wisconsin  
*Inga Hofmann, MD*

Medical College of Wisconsin  
Hematologic Malignancies Program  
Milwaukee, Wisconsin  
*Ehab Atallah, MD*

University of Wisconsin, Madison Medical School  
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*Ryan Mattison, MD*

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Vienna, Austria  
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Vienna, Austria  
*Peter Valent, MD*

University Hospital of Innsbruck  
Innsbruck, Austria  
*Reinhard Stauder, MD*

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*Michel Delforge, MD, PhD*

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Hospital das clínicas da Faculdade de Medicina  
da Universidade de São Paulo  
São Paulo, Brazil  
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Universidade Federal de Ceará  
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Universidade Federal de São Paulo  
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Odette Cancer Centre  
Sunnybrook Health Sciences Center  
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Guangdong Academy of Medical Sciences  
Guangzhou, China  
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Institute of Hematology and Blood Diseases Hospital  
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Tianjin, China  
*Zhijian Xiao, MD*

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Jiangsu Province, China  
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The First Affiliated Hospital of Zhejiang University  
Zhejiang Province, China  
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Seoul, Korea  
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Yonsei University College of Medicine  
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Karolinska University Hospital Huddinge  
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Eva Hellström-Lindberg, MD, PhD

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Lee-Yung Shih, MD

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Osman Ilhan, MD

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Research Center for Radiation Medicine  
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Aberdeen University School of Medicine  
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Mike Dennis, MD/Dan Wiseman, MD  
King's College London &  
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University Hospital Birmingham NHS Trust  
Birmingham, United Kingdom  
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Radcliffe Hospitals & University of Oxford  
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Paresh Vyas, MD  
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St. James's Institute of Oncology  
Leeds, United Kingdom  
Catherine Cargo, MD  
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Christopher Dalley, MD  
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Cardiff, Wales  
Jonathan Kell, MD  
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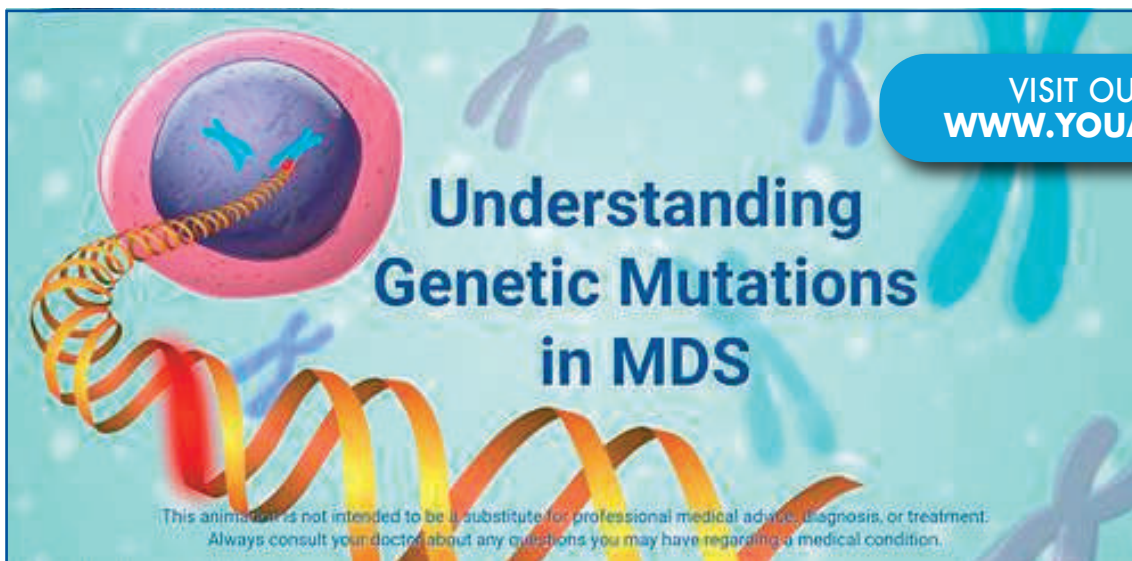
## PATIENT RESOURCES

**HAVE YOU CHECKED OUT OUR YOU AND MDS ANIMATED PATIENT VIDEO SERIES YET?**

**NEW 'YOU AND MDS' MODULE ALERT:**

**UNDERSTANDING GENETIC MUTATIONS IN MYELODYSPLASTIC SYNDROMES (MDS)**

This brand-new animated learning resource is the newest addition to our *You and MDS* series. It is intended for patients with MDS, as well as family members and caregivers. Learn about genetic changes in myelodysplastic syndromes (MDS), and the many different driver mutations that are associated with MDS. Some mutated genes are associated with lower-risk disease, while others may indicate greater risk. Your mutation profile can change over time, so it is important to repeat the testing at different stages of your treatment. The more you know about your genetic makeup in MDS, the more you will understand the outlook and, in some cases, the treatment that is most likely to be effective.



## THE ADDED BENEFITS OF CLINICAL TRIALS

*If you are diagnosed with MDS, participating in a clinical trial may offer you a number of advantages in addition to the standard treatment. Through our partnership with our MDS Centers of Excellence and industry partners, patients have access to the latest clinical trials on the MDSF website here <https://www.mds-foundation.org/clinical-trial-announcements/>.*

**POTENTIAL BENEFITS INCLUDE:**

- Working with top specialists who conduct research and are highly knowledgeable about the latest treatments.
- Being offered cutting-edge treatments not yet available to the general population that may help you live longer and/or improve your quality of life.
- Playing a meaningful role in a study that could help other patients in the future.

# Your insights can change the future of cancer support

Your cancer experience can inspire change

Start Survey

## Cancer Experience Registry Survey

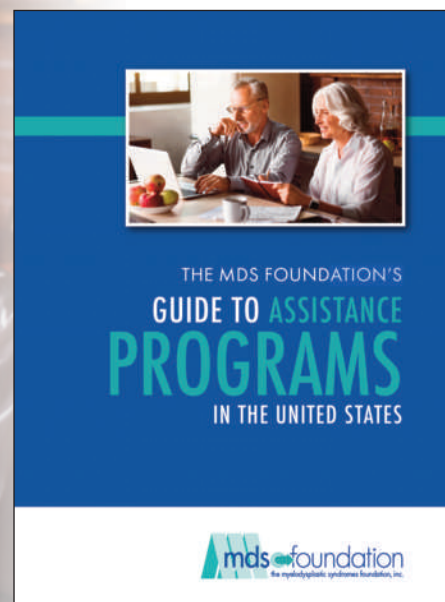
We are excited to join forces with Cancer Support Community to share their newly launched MDS Cancer Experience Registry (CER).

The Cancer Experience Registry is a free and confidential online survey for anyone who has ever been diagnosed with cancer, and for caregivers of individuals with cancer, to share their cancer experience. The findings gathered from these surveys will illustrate the Cancer Support Community's commitment to putting the voices of patients and caregivers at the center of the conversation about cancer.

By taking the survey, you join thousands of others in helping to: influence health care policies, enhance cancer care, and improve support services.

***Join today and elevate your voice!***

<https://www.cancersupportcommunity.org/registry28>



Please visit our website:

<https://www.mds-foundation.org/wp-content/uploads/2022/02/LARGE-MDS-Insurance-Booklet-PRINT.pdf>

## GUIDE TO ASSISTANCE PROGRAMS IN THE UNITED STATES

***We have assembled a listing of assistance programs available to MDS patients. It is important to know that there is support for those who cannot afford medicine or other healthcare costs. We hope this new resource will be beneficial in helping you with your medical needs.***

## ORGANIZE A FUNDRAISER

**Are you interested in learning more about hosting a fundraiser on behalf of the MDS Foundation?**

**If so, we invite you to check out our newly launched Fundraiser Toolkit:**

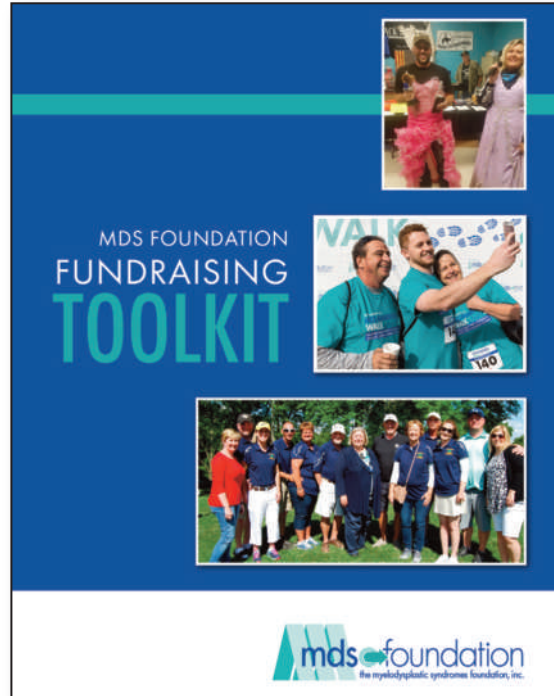
**<https://bit.ly/3Kx4B8e>**

We're providing you with the resources and inspiration to get started on hosting an MDS fundraiser and benefiting patients and their loved ones. Every dollar counts. Thank you for your support!

In 2022, we held five **Move for MDS** walks that took place in Los Angeles, Chicago, Nashville, NYC, and Boston/Global and we are thrilled to share that we have raised over

**raised over \$375,000**

to accelerate critical research of Myelodysplastic Syndromes.



**We already consider you part of the family...  
NOW, LET'S BE FRIENDS!!**

FIND US ON FACEBOOK



Like us on  
**Facebook**

FOLLOW US ON TWITTER



@MDSFoundation



[www.youtube.com/c/MDSFoundationInc](http://www.youtube.com/c/MDSFoundationInc)



[www.linkedin.com/  
company/mds-foundation-inc](http://www.linkedin.com/company/mds-foundation-inc)



[www.instagram.com/  
mdsfoundation/](http://www.instagram.com/mdsfoundation/)

## GREAT DEALS. GOOD DEEDS.

Transform every online purchase into a donation for the Myelodysplastic Syndromes Foundation

<https://givingassistant.org/np#myelodysplastic-syndromes-foundation>

Use **Giving Assistant** to save money and support the Myelodysplastic Syndromes (MDS) Foundation, Inc. It's free to help the MDS Foundation when you sign up for **Giving Assistant** and shop at Bed Bath & Beyond, Aliexpress, and ULTA! Sign up today and get donating!

<https://smile.amazon.com>

CHARITY: Myelodysplastic Syndromes Foundation

To shop at **AmazonSmile** simply go to smile.amazon.com on your web browser or activate AmazonSmile on your Amazon Shopping app on your iOS or Android phone (found under settings on your app). **AmazonSmile** will donate 0.5% of your eligible purchases to the MDS Foundation.

<https://www.igive.com>

**iGive** automatically helps your favorite cause every time you shop. Use **iGive** to donate a percentage of your online shopping to the MDSF. Choose the MDS Foundation and you'll earn money for free!



## PLANNED GIVING LEAVING A LEGACY...

### WRITE THE MDSF INTO YOUR WILL

In addition to the gifts you give today and throughout your lifetime, taking the time to write MDSF into your will—or to make any other planned/estate gift—provides an enduring legacy of your personal interest and commitment to providing education, service, and research for those facing bone marrow failure diseases. Ask your attorney to include this paragraph, specified to your gift preferences, in your will:



*I give, devise, and bequeath \$\_\_\_\_ (amount) or \_\_\_\_% (percentage) to the MDS Foundation, 4573 South Broad Street, Suite 150, Yardville, NJ 08620, a not-for-profit corporation for its charitable uses as directed by its Board of Directors.*

It is important to remember your friends and family when drawing up a will and to make sure that all loved ones are taken care of. Once you have done this, you may wish to leave a legacy to the MDS Foundation. Leaving a legacy to the MDS Foundation is one of the greatest gifts that you can give.

JOIN THE MDS FOUNDATION

# A NEW PARTNERSHIP



## OFFICIAL JOURNALS OF THE MDS FOUNDATION

*Leukemia Research Reports*

*Leukemia Research*

*Clinical and Laboratory Studies*



**60%** MDSF Professional Members receive a **SUBSCRIPTION DISCOUNT – ANNUALLY**

## MDS PROFESSIONAL MEMBERSHIP OPTIONS – JOIN NOW

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

### **\$50** **Community Professional Membership**

Includes discounted registration rates at MDSF meetings, 60% annual subscription discount to *Leukemia Research*, as well as access to MDSF resources for distribution to your patients.

### **\$250** **Change the Future of MDS Professional Membership**

Change the Future of MDS Professional Membership (Includes discounted registration rates at MDSF meetings, 60% annual subscription discount to *Leukemia Research*, access to MDSF resources for distribution to your patients, as well as the opportunity to present at MDSF patient events in your region). In addition, \$50 of your membership will help support a Professional outside of the United States that represents a CoE in financial need. Member names are listed on the MDSF website.



## 2023 PATIENT FORUMS

PATIENT FORUMS

### IN-PERSON LIVING WITH MDS FORUMS ARE BACK!



STAY TUNED FOR OUR ONGOING MEETINGS IN THE US AND EUROPE PLANNED FOR 2023  
PLEASE MAKE SURE TO REGULARLY CHECK OUR WEBSITE AND FACEBOOK FOR DETAILS COMING SOON.

We are happy to announce that we are resuming our in-person patient and caregiver forums. The diagnosis of MDS is often unexpected and filled with both immediate and long-term challenges. You probably have many questions. Learn the latest on the diagnosis and treatment of MDS from leading experts in the field.

**WHETHER YOU ARE A NEWLY DIAGNOSED PATIENT, A LONG-TERM SURVIVOR, OR CAREGIVER, OUR FREE ONE-DAY LIVE IN-PERSON FORUMS WILL HAVE SOMETHING FOR YOU!**

**LEARN MORE AT: [www.mds-foundation.org/patient-and-family-forums](http://www.mds-foundation.org/patient-and-family-forums)**

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

**DON'T MISS OUT ON THESE INFORMATIVE, FREE EVENTS**

## DOWNLOAD OUR MOBILE APP!

### MDS FOUNDATION APP

HAVE MDS INFORMATION AT YOUR FINGERTIPS!

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

Available in the Google Play Store and iTunes





## 2023 WEBINARS COMING SOON!

# WE ARE VIRTUAL!



The MDS Foundation will be hosting a series of webinars bringing renowned experts to your computer using easy to understand language in a 90-minute format. We will be collaborating with world renowned hematology professionals who will address key topics including live Q&A opportunities for all participants.

**PLEASE MAKE SURE TO REGULARLY CHECK OUR WEBSITE AND FACEBOOK FOR DETAILS COMING SOON.**



<https://www.mds-foundation.org/2022-webinars-for-mds-patients-caregivers>



## SUBSCRIBE: MDS FOUNDATION PODCASTS

**THIS PODCAST SERIES PROVIDES IMPORTANT UP-TO-THE-MINUTE INFORMATION ON MDS INCLUDING DIAGNOSIS, TREATMENT AND CLINICAL RESEARCH.**

The explosion of information on MDS forces us to seek novel, alternative ways to distribute it. Podcasts gives us an easy and popular way to communicate this information in a short time.

### SUBSCRIBE!



#### MDS PROFESSIONAL REPORT

##### SEASON 2: EPISODE 2: *The Role of Genetics in MDS Management*

*Drs. Rafael Bejar (San Diego) and Moshe Mittelman (Tel Aviv) discuss several papers highlighting the role of genetics in MDS diagnosis, follow up and prediction of treatment. They also discuss the role of the newly approved luspatercept in the treatment of anemic transfusion-dependent patients with lower-risk MDS.*



#### MDS PATIENT & FAMILY REPORT

*This new initiative of the MDS Foundation is devoted to MDS patients, family members and caregivers. In each episode, experts in the field will discuss novel information on MDS, such as new diagnostic techniques, new therapies etc. They will also answer frequently asked questions.*

##### SEASON 1: EPISODE 1: *MDS is Already in the Genetic Era*

*The first episode of this program is a conversation between Prof. Guillermo Sanz from Valencia, Spain and Prof. Moshe Mittelman from Tel Aviv, Israel, discussing several issues relevant for patients, families and other stakeholders interested in myelodysplastic syndromes.*

##### SEASON 1: EPISODE 2: *Personalized Treatment of MDS*

*Drs. Rafael Bejar (San Diego) and Moshe Mittelman (Tel Aviv) discuss the trend towards adjusting the appropriate treatment to the particular MDS patient, a trend that is associated with higher rate of successful treatments and less toxicity. They also address several frequently asked questions.*



## BRISTOL MYERS SQUIBB LAUNCHES DISABILITY DIVERSITY IN CLINICAL TRIALS (DDICT) INITIATIVE TO IMPROVE HEALTHCARE OUTCOMES FOR PEOPLE WITH DISABILITIES

**PRINCETON, NJ. JULY 22, 2022 (BUSINESS WIRE).** Bristol Myers Squibb (NYSE:BMJ) today announced, in collaboration with Disability Solutions, a U.S.-based non-profit organization that supports companies globally to achieve true disability inclusion, the launch of the Disability Diversity in Clinical Trials (DDiCT) initiative. This new initiative aligns with Bristol Myers Squibb's broader inclusion and diversity health equity commitments to address health disparities, clinical trial diversity, supplier diversity, employee giving, and workforce representation between 2020 and 2025.

The DDiCT initiative initially aims to make recommendations on how to effectively improve access, engagement, speed of enrollment, and participation of people with disabilities in clinical trials, to ensure all patient groups are reflective of the real-world population and aligned with the epidemiology of the disease studies. This project was initiated by the Bristol Myers Squibb People & Business Resource Group DAWN (Disability Advancement Workplace Network) and will be co-led by DAWN and the Global Drug Development Team.

"Through this work, Bristol Myers Squibb can set the standard and stage for access to life-changing and life-saving medicines for people with disabilities," said Samit Hirawat, M.D., executive vice president and chief medical officer, Global Drug Development, Bristol Myers Squibb. "The long-term goal of our DDiCT program is to develop and pilot trials that are accessible to the widest variety of patients."

Current common clinical trial practices exclude up to one-fourth of the U.S. population-based on disability status. According to a study published in the Journal of the American Medical Association, in 338 phase III and IV studies, 12.4% of people with intellectual or developmental disabilities and

1.8% of those with physical disabilities failed to be included due to explicit exclusion criteria. The study also identified that additional barriers for disability diversity within clinical trials are caused by inaccessible trial sites, medical equipment, and ableist biases which deter this community from receiving potentially life-saving treatments.

"People with disabilities are omitted from conversations about diversity and inclusion, despite being the largest underrepresented group in the world and the only underrepresented group anyone can join at any given moment. Therefore, it's essential that we broaden the scope of medical trials and research," said Tinamarie Duff, DAWN Global People & Business Resource Group Lead, Bristol Myers Squibb. "The launch of the DDiCT, especially during Disability Pride Month, supports Bristol Myers Squibb's overall commitment to address every dimension of diversity, which means making the most effective medicine to include people with disabilities at all stages of access/trials."

### ABOUT BRISTOL MYERS SQUIBB

Bristol Myers Squibb is a global biopharmaceutical company whose mission is to discover, develop and deliver innovative medicines that help patients prevail over serious diseases. For more information about Bristol Myers Squibb, visit us at BMS.com or follow us LinkedIn, Twitter, YouTube, Facebook, and Instagram.

### ABOUT DISABILITY SOLUTIONS

Disability Solutions is a division of Ability Beyond and works directly with forward thinking and inclusive companies who have identified the value of being inclusive of people with disabilities in all facets of their organization. Disability Solutions works with more than 60 companies globally to drive true disability inclusion. Their motto is "Changing Minds and Changing Lives". Disability Solutions understands that when corporate leaders engage with the value and humanity the disability community brings forward, they will be able to drive life-changing — and in this case — life-saving change which will echo across the world. You can learn more about Disability Solutions at [www.disabilitytalent.org](http://www.disabilitytalent.org).

About Disability Solutions Parent Company: Ability Beyond, Inc. is a nonprofit 501(c)3 organization whose mission is to provide health and human services to adults with disabilities throughout Connecticut and Westchester County, NY. Founded more than 60 years ago, Ability Beyond has its headquarters in Bethel, CT, and Chappaqua, NY. Ability Beyond employs 1,200 people and has an annual budget of \$71,000,000. Jane Davis, President/CEO, and her Executive Team manage the agency, with oversight from the Ability Beyond Board of Directors. Major revenue sources include Medicaid, Medicare, government contracts, fees for service, and philanthropic support. You can learn more about Ability Beyond at [www.abilitybeyond.org](http://www.abilitybeyond.org).

## KARYOPHARM GRANTED REGULATORY DESIGNATIONS FOR ELTANEXOR FOR THE TREATMENT OF MYELODYSPLASTIC SYNDROMES

**NEWTON, MA. JULY 20, 2022 (PRNewswire)**

*FDA Fast Track Designation and European Commission Orphan Medicinal Product Designation*

Karyopharm Therapeutics Inc. (Nasdaq: KPTI), a commercial-stage pharmaceutical company pioneering novel cancer therapies, today announced new regulatory designations for eltanexor, a novel oral, Selective Inhibitor of Nuclear Export (SINE) investigational compound being studied for the treatment of myelodysplastic syndromes (MDS): (i) the U.S. Food and Drug Administration (FDA) has granted fast track designation for the development program of eltanexor as monotherapy for the treatment of patients with relapsed or refractory intermediate, high-, or very high-risk MDS; (ii) the European Commission (EC) adopted the Committee for Orphan Medicinal Products (COMP) opinion to designate eltanexor as an orphan medicinal product for the treatment of MDS in the European Union (EU). Karyopharm

also received orphan drug designation from the FDA in January 2022. MDS are a group of diseases characterized by ineffective production of the components of the blood due to poor bone marrow function with a risk of progression to acute myeloid leukemia.

Karyopharm is currently investigating eltanexor in an ongoing open-label Phase 1/2 study in patients with relapsed/refractory MDS. Previously, Karyopharm reported initial data from the Phase 1 portion of this study evaluating single-agent eltanexor in patients with hypomethylating agent (HMA)-refractory MDS.

Approximately 15,000 people in the U.S. and 14,000 people in the EU are expected to be diagnosed with intermediate-to-high risk MDS in 2022. HMAs are the current standard of care for newly diagnosed, higher-risk MDS patients. However, only 40-60% of patients respond, with these responses typically lasting less than two years. The prognosis in HMA-refractory disease is poor, with a median overall survival of four to six months. There are currently no approved therapies for HMA- refractory MDS.

“These recent designations from the FDA and EC reinforce eltanexor's potential to improve clinical outcomes for patients with relapsed/ refractory MDS,” said Richard Paulson, President and Chief Executive Officer of Karyopharm. “We are dedicated to advancing our ongoing clinical trials and remain committed to bringing eltanexor to these patients and their families as a new treatment option.”

Fast track is a process designed by the FDA to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions. Once a drug receives Fast Track designation, early and frequent communication between the FDA and the drug company is encouraged throughout the entire drug development and review process.

Orphan Medicinal Product Designation is granted by the EC to promote the development of drugs that target rare (less than 5 in 100,000 people across the EU), seriously

debilitating and/or life-threatening diseases, and are expected to provide a significant benefit over existing authorized treatments. Orphan designation qualifies a company for certain incentives that apply across all stages of drug development, including the potential for ten years of market exclusivity following marketing approval, fee reductions, and eligibility for orphan drug grants.

#### ABOUT ELTANEXOR

Eltanexor (KPT-8602) is an investigational novel SINE compound that functions by binding with, and inhibiting, the nuclear export protein, XPO1, leading to the accumulation of tumor suppressor proteins in the cell nucleus. This reinitiates and amplifies their tumor suppressor function and is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells.

In preclinical models, eltanexor has a broad therapeutic window with minimal penetration of the blood brain barrier and, therefore, has the potential to serve as another SINE compound for cancer indications. Following oral administration, animals treated with eltanexor show lower percentage of body weight loss and improved food consumption than animals similarly treated with selinexor. This allows more frequent dosing of eltanexor, enabling a longer period of exposure than is possible with selinexor.

Eltanexor is an investigational medicine and has not been approved by the United States Food and Drug Administration or any other regulatory agency.

#### ABOUT KARYOPHARM THERAPEUTICS

Karyopharm Therapeutics Inc. (Nasdaq: KPTI) is a commercial-stage pharmaceutical company pioneering novel cancer therapies. Since its founding, Karyopharm has been the industry leader in oral Selective Inhibitor of Nuclear Export (SINE) compound technology, which was developed to address a fundamental mechanism of oncogenesis: nuclear export dysregulation. Karyopharm's lead SINE compound and first-in-class, oral exportin 1 (XPO1) inhibitor, XPOVIO® (selinexor), is approved in the U.S. and marketed by the Company in three oncology indications and has received regulatory

approvals in various indications in a growing number of ex-U.S. territories and countries, including Europe and the United Kingdom (as NEXPOVIO®) and China. Karyopharm has a focused pipeline targeting multiple high unmet need cancer indications, including in multiple myeloma, endometrial cancer, myelodysplastic syndromes and myelofibrosis. For more information about our people, science and pipeline, please visit [www.karyopharm.com](http://www.karyopharm.com), and follow us on Twitter at @Karyopharm and LinkedIn.

### SYROS RECEIVES POSITIVE OPINION ON ORPHAN DRUG DESIGNATION FROM THE EUROPEAN MEDICINES AGENCY FOR TAMIBAROTENE FOR THE TREATMENT OF MDS

**CAMBRIDGE, MA. AUGUST 03, 2022 (BUSINESS WIRE).** Syros Pharmaceuticals (NASDAQ:SYRS), a leader in the development of medicines that control the expression of genes, today announced that the European Medicines Agency (EMA) issued a positive opinion on the Company's application for orphan drug designation for tamibarotene for the treatment of myelodysplastic syndrome (MDS). Tamibarotene, an oral first-in-class selective retinoic acid receptor alpha (RAR $\alpha$ ) agonist, is currently being evaluated in combination with azacitidine in the SELECT-MDS-1 Phase 3 trial for RARA-positive patients with newly diagnosed higher-risk MDS (HR-MDS).

Previously, the U.S. Food and Drug Administration (FDA) granted orphan drug designation to tamibarotene in MDS in February 2022.

“We are pleased that the EMA has issued a positive opinion for orphan drug designation for tamibarotene as it represents an important milestone for MDS patients, who have an urgent need for effective, tolerable, and convenient treatment options,” said David A. Roth, M.D., Syros' Chief Medical Officer. “We believe tamibarotene has the potential to change the current standard of care and become the first therapy for a targeted population in HR-MDS. We continue

to advance our SELECT-MDS-1 trial and are looking forward to announcing pivotal data in late 2023 or early 2024.”

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion issued by the EMA Committee for Orphan Medicinal Products. The EMA’s orphan designation is available to companies developing treatments for life-threatening or chronically debilitating conditions that affect fewer than five in 10,000 persons in the EU. Medicines that meet the EMA’s orphan designation criteria qualify for financial and regulatory incentives that include a 10-year period of marketing exclusivity in the EU after product approval, protocol assistance from the EMA at reduced fees during the product development phase and access to centralized marketing authorization.

The ongoing SELECT-MDS-1 Phase 3 clinical trial is evaluating the safety and efficacy of tamibarotene in combination with azacitidine for RARA-positive patients with newly diagnosed HR-MDS. Data from the pivotal trial are expected in the fourth quarter of 2023 or the first quarter of 2024, with a potential new drug application filing expected in 2024.

Syros is also evaluating tamibarotene in combination with azacitidine and venetoclax for RARA-positive patients with newly diagnosed unfit acute myeloid leukemia (AML), for which tamibarotene had previously received orphan drug designation from both the FDA and EMA. Data from the safety lead-in portion of the SELECT-AML-1 Phase 2 trial is expected in the second half of this year.

### ABOUT SYROS PHARMACEUTICALS

Syros is redefining the power of small molecules to control the expression of genes. Based on its unique ability to elucidate regulatory regions of the genome, Syros aims to develop medicines that provide a profound benefit for patients with diseases that have eluded other genomics-based approaches. Syros is advancing a robust clinical-stage pipeline, including: tamibarotene, a first-in-class oral selective RAR agonist in RARA-positive patients with higher-risk myelodysplastic syndrome and acute myeloid

leukemia; SY-2101, a novel oral form of arsenic trioxide in patients with acute promyelocytic leukemia; and SY-5609, a highly selective and potent oral CDK7 inhibitor in patients with select solid tumors. Syros also has multiple preclinical and discovery programs in oncology and monogenic diseases. For more information, visit [www.syros.com](http://www.syros.com) and follow us on Twitter (@SyrosPharma) and LinkedIn.

## GAMIDA CELL ANNOUNCES FDA ACCEPTANCE OF BIOLOGICS LICENSE APPLICATION FOR Omidubicel WITH PRIORITY REVIEW

**BOSTON, MA. AUGUST 01, 2022 (BUSINESS WIRE).** Gamida Cell Ltd. (Nasdaq: GMDA), the leader in the development of NAM-enabled cell therapy candidates for patients with hematologic and solid cancers and other serious diseases, announced today that the U.S. Food and Drug Administration (FDA) has accepted for filing the Company’s Biologics License Application (BLA) for omidubicel for the treatment of patients with blood cancers in need of an allogeneic hematopoietic stem cell transplant. Omidubicel is a first-in-class, advanced NAM-enabled stem cell therapy candidate with breakthrough and orphan drug designations.

The FDA granted Priority Review for the BLA and has set a Prescription Drug User Fee Act (PDUFA) target action date of January 30, 2023. The FDA grants Priority Review to product applications that, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. At this time, the FDA has indicated that it is not planning an advisory committee meeting as part of the BLA review.

“The FDA’s acceptance of our BLA with Priority Review signifies a critical milestone in our mission to deliver a new stem cell therapy option for patients in need of a donor for an

allogeneic stem cell transplant,” said Julian Adams, Ph.D., chief executive officer of Gamida Cell. “We are encouraged by the positive and sustained follow-up results from patients participating in the Phase 3 trial of omidubicel, including a positive overall survival trend one-year out from treatment. These results provide promising rationale that, if approved, omidubicel could become a treatment of choice for patients in need of an allo-HSCT transplant. We look forward to working with the FDA throughout the review process to bring omidubicel to patients as quickly as possible.”

Upon FDA approval, omidubicel will be manufactured at the Gamida Cell owned manufacturing facility in Israel. This is a newly constructed, state-of-the-art, modular facility which allows for additional capacity to be added to address growing demand. Batches from this facility were used to support the BLA for omidubicel and the facility is currently manufacturing clinical batches.

The omidubicel BLA is supported by the statistically significant results from Gamida Cell’s pivotal Phase 3 study, the results of which were published in *Blood*, the official journal of the American Society of Hematology. Results for the study’s primary endpoint, the median time to neutrophil engraftment in patients with hematologic malignancies undergoing allogeneic bone marrow transplant with omidubicel compared to standard umbilical cord blood (UCB), demonstrated a median time to neutrophil engraftment of 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ( $p < 0.001$ ). The secondary endpoints of this Phase 3 study were all achieved and were statistically significant. These secondary endpoints were platelet engraftment, the rate of infection, and days alive and out of hospital. Omidubicel was generally well tolerated in the Phase 3 study.

In 2019, approximately 8,000 patients who were 12 years old and up with hematologic malignancies underwent an allogeneic stem cell transplant in the United States. Unfortunately, it is estimated that another 1,200 patients were eligible for transplant but could not find a donor source. If approved, omidubicel has the potential to improve outcomes for patients based on

transplanter feedback and to potentially increase access for patients to get to transplant. If approved, omidubichel has the potential to treat approximately 2,000–2,500 patients each year in the U.S.

#### ABOUT OmidUBICEL

Omidubichel is an advanced cell therapy candidate developed as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplant for patients with blood cancers. Omidubichel demonstrated a statistically significant reduction in time to neutrophil engraftment in comparison to standard umbilical cord blood in an international, multi-center, randomized Phase 3 study (NCT0273029) in patients with hematologic malignancies undergoing allogeneic bone marrow transplant. The Phase 3 study also showed reduced time to platelet engraftment, reduced infections and fewer days of hospitalization. One-year post-transplant data showed sustained clinical benefits with omidubichel as demonstrated by significant reduction in infectious complications as well as reduced non-relapse mortality and no significant increase in relapse rates nor increases in graft-versus-host-disease (GvHD) rates. Omidubichel is the first stem cell transplant donor source to receive Breakthrough Therapy Designation from the FDA and has also received Orphan Drug Designation in the US and EU. Omidubichel is an investigational stem cell therapy candidate, and its safety and efficacy have not been established by the FDA or any other health authority. For more information about omidubichel, please visit

#### ABOUT NAM TECHNOLOGY

Our NAM-enabling technology is designed to enhance the number and functionality of targeted cells, enabling us to pursue a curative approach that moves beyond what is possible with existing therapies. Leveraging the unique properties of NAM (nicotinamide), we can expand and metabolically modulate multiple cell types — including stem cells and natural killer cells — with appropriate growth factors to maintain the cells' active phenotype and enhance potency. Additionally, our NAM technology improves the metabolic fitness of cells, allowing for continued activity throughout the expansion process.

#### ABOUT GAMIDA CELL

Gamida Cell is pioneering a diverse immunotherapy pipeline of potentially curative cell therapy candidates for patients with solid tumor and blood cancers and other serious blood diseases. We apply a proprietary expansion platform leveraging the properties of NAM to allogeneic cell sources including umbilical cord blood-derived cells and NK cells to create therapy candidates with potential to redefine standards of care. These include omidubichel, an investigational product with potential as a life-saving alternative for patients in need of bone marrow transplant, and a line of modified and unmodified NAM-enabled NK cells targeted at solid tumor and hematological malignancies. For additional information, please visit [cell.com](http://cell.com) or follow Gamida Cell on LinkedIn, Twitter, Facebook Cautionary Note Regarding Forward Looking Statements or Instagram at @GamidaCellTx.

### MORPHOSYS PRESENTS MULTIPLE ANALYSES OF THE MANIFEST PHASE 2 TRIAL INVESTIGATING THE POTENTIAL OF PELABRESIB IN THE TREATMENT OF

*MorphoSys Presents Multiple Analyses of the MANIFEST Phase 2 Trial Investigating the Potential of Pelabresib in the Treatment of Myelofibrosis at EHA 2022*

**PLANEGG/MUNICH, GERMANY  
OCTOBER 6, 2022**

- *New translational data suggests potential disease-modifying effects following treatment with pelabresib of both first-line and ruxolitinib-relapsed/refractory patients*
- *A comparative model suggests an improvement in SVR35 and TSS50 in JAK inhibitor-naïve myelofibrosis patients treated with pelabresib plus ruxolitinib over JAK inhibitors as monotherapy*
- *Additional presentations include positive interim data from the MANIFEST Phase 2 trial and design of MANIFEST-2, a global*

*Phase 3, randomized, double-blind trial of pelabresib in combination with ruxolitinib in treatment-naïve patients*

MorphoSys AG (FSE: MOR; NASDAQ: MOR) is presenting data from multiple analyses of the ongoing MANIFEST study, an open-label Phase 2 clinical trial of pelabresib, an investigational BET inhibitor, in patients with myelofibrosis, a rare bone marrow cancer for which only limited treatment options are available. The latest findings suggest pelabresib may have disease-modifying properties and confirm previous data supporting the potential of pelabresib as a treatment for patients with myelofibrosis. The data are being presented during oral and poster sessions at the European Hematology Association 2022 (EHA 2022) Hybrid Congress being held in Vienna.

“The standard for evaluating disease response in myelofibrosis focuses on symptom relief rather than true disease modification, which remains an unmet need for these patients,” said John Mascarenhas, M.D., Director of the Adult Leukemia Program at The Tisch Cancer Institute at Mount Sinai, New York. “The body of data being presented at EHA 2022 — including new findings that pelabresib may address cellular defects seen in myelofibrosis, thereby getting at the root cause of the disease — with correlated clinical improvements, suggests pelabresib may have the potential to enhance the current standard of care in the first-line treatment of myelofibrosis.”

A study that will be presented in an oral session on June 11 analyzed cells derived from blood of patients who enrolled in the MANIFEST trial and from healthy volunteers. The findings indicate that pelabresib alone or in combination with the JAK inhibitor ruxolitinib may have the potential to improve the typical imbalance in the two white blood cell populations, the myeloid and lymphoid cells, and help restore normal blood cell development. These improvements also correlated with decreases in spleen volume, a key clinical measure of treatment success. Additionally, pelabresib alone or in combination decreased pro-inflammatory and pro-fibrotic signaling in monocytes, suggesting a potential attenuation of disease processes.

“The latest findings from the MANIFEST trial at EHA 2022 highlight the potential of pelabresib to offer patients and their physicians benefits over monotherapy with JAK inhibitors, if approved,” said Malte Peters, M.D., MorphoSys Chief Research and Development Officer. “The full complement of MANIFEST data being presented this week suggests pelabresib may help improve outcomes for patients with myelofibrosis and reaffirms our confidence in the Phase 3 MANIFEST-2 study. We are committed to these patients, who need better options in first-line treatment and beyond.”

A second oral presentation on June 11 highlights positive interim data from the MANIFEST trial on the safety and efficacy of pelabresib in combination with ruxolitinib in patients who were not previously treated with a JAK inhibitor and in those with suboptimal response to ruxolitinib. The findings show that the combination was generally well tolerated and offered reductions in spleen volume and symptom burden, with disease-modifying activity as measured by reduced levels of pro-inflammatory cytokines and improved bone marrow morphology. Over two-thirds (68%; n=57) of JAK inhibitor-naïve patients treated with the combination achieved at least a 35% reduction in spleen volume (SVR35) from baseline at week 24. Notably, 80% of patients achieved SVR35 at any time on study. Most patients also saw their symptoms reduced, with 56% (n=46) achieving at least a 50% reduction in total symptom score (TSS50) from baseline at week 24. No new safety signals were identified in the study. The most common hematologic adverse events were thrombocytopenia (12%, grade 3/4) and anemia (34%, grade 3/4). Non-hematological events included dyspnea (5%, grade 3) and respiratory tract infections (8%, grade 3/4).

In a poster presentation at EHA 2022, matching-adjusted indirect comparisons were used to compare findings for the combination of pelabresib plus ruxolitinib in treatment-naïve patients with intermediate- or high-risk disease in one arm of the MANIFEST trial with findings from historical clinical trials examining the use of JAK inhibitor monotherapy in myelofibrosis. Adjusting for

cross-trial differences, the estimated response rate ratios favored the pelabresib combination over ruxolitinib, fedratinib or momelotinib monotherapy for SVR35 and for TSS50, suggesting improved efficacy versus the JAK inhibitors alone.

A second poster presentation includes trial design information for the Phase 3 MANIFEST-2 study. MANIFEST-2, which is currently enrolling, will compare pelabresib in combination with ruxolitinib versus placebo plus ruxolitinib in approximately 400 patients with myelofibrosis who are naïve to JAK inhibitor therapy. MorphoSys is expected to report topline data from the MANIFEST-2 trial in the first half of 2024.

#### ABOUT PELABRESIB

Pelabresib (CPI-0610) is an investigational selective small molecule designed to promote anti-tumor activity by inhibiting the function of bromodomain and extra-terminal domain (BET) proteins to decrease the expression of abnormally expressed genes in cancer. Pelabresib is being investigated as a treatment for myelofibrosis and has not yet been evaluated or approved by any regulatory authorities.

#### ABOUT MYELOFIBROSIS

Myelofibrosis is a type of chronic leukemia that causes extensive scarring in the bone marrow, which disrupts the body’s normal production of healthy blood cells. The result is a reduction in red blood cells, which can cause weakness and fatigue, and in platelets, which increases the risk of bleeding due to deficient clotting. Myelofibrosis often causes an enlarged spleen. It is most often diagnosed in people older than 50 and can occur on its own (called primary myelofibrosis) or because of another bone marrow disorder.

#### ABOUT MANIFEST

MANIFEST (NCT02158858) is an open-label Phase 2 clinical trial of pelabresib in patients with myelofibrosis.

The MANIFEST trial is evaluating pelabresib in combination with ruxolitinib in JAK-inhibitor-naïve myelofibrosis patients (Arm 3), with a primary endpoint of the proportion of patients with a  $\geq 35\%$  spleen volume reduction from baseline (SVR35) after 24 weeks of

treatment. The trial is also evaluating pelabresib either as a monotherapy in patients who are resistant to, intolerant of, or ineligible for ruxolitinib and no longer on the drug (Arm 1) or as add-on therapy in combination with ruxolitinib in patients with a suboptimal response to ruxolitinib or myelofibrosis progression (Arm 2). Patients in Arms 1 and 2 are being stratified based on transfusion-dependent (TD) status. The primary endpoint for the patients in cohorts 1A and 2A, who were TD at baseline, is conversion to transfusion independence for 12 consecutive weeks. The primary endpoint for patients in cohorts 1B and 2B, who were not TD at baseline, is the proportion of patients with a  $\geq 35\%$  spleen volume reduction from baseline after 24 weeks of treatment.

Constellation Pharmaceuticals, Inc., a MorphoSys company, is the MANIFEST trial sponsor.

#### ABOUT MANIFEST-2

MANIFEST-2 (NCT04603495) is a global, double-blind, randomized Phase 3 clinical trial with pelabresib in combination with ruxolitinib versus placebo plus ruxolitinib in JAK inhibitor-naïve patients with myelofibrosis. The primary endpoint of the study is a 35% or greater reduction in spleen volume (SVR35) from baseline at 24 weeks. A key secondary endpoint of the study is a 50% or greater improvement in total symptom score (TSS50) from baseline at 24 weeks.

Constellation Pharmaceuticals, Inc., a MorphoSys company, is the MANIFEST-2 trial sponsor.

#### ABOUT MORPHOSYS

At MorphoSys, we are driven by our mission: More life for people with cancer. As a global commercial-stage biopharmaceutical company, we use groundbreaking science and technologies to discover, develop, and deliver innovative cancer medicines to patients. MorphoSys is headquartered in Planegg, Germany, and has its U.S. operations anchored in Boston, Massachusetts. To learn more, visit us at [www.morphosys.com](http://www.morphosys.com) and follow us on Twitter and LinkedIn.

### MY DIAGNOSIS STORY

**MICHELLE WILSON**

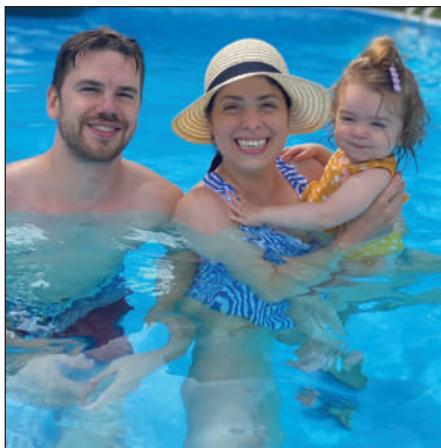
*West Milford, New Jersey*

I underwent a bone marrow biopsy on August 23, 2021.

Two days later, on August 25, 2021, I received my diagnosis. This is my story.

My mom and I arrived at the appointment early, as I was eager, anxious, and ready to get it over with. Little did I know, this would be just the beginning. The nurse called us in; we walked down the hallway and into the exam room, and I sat down on the exam table. The doctor walked in, sat down on the stool, and immediately began the conversation. In a manner of shock, Dr. P looked at me and said, "Well, you have myelodysplastic syndrome (MDS)." She said this as if she was processing it herself. I looked at her without any reaction and said "Okay..." because I hadn't heard of it before. I swallowed the lump in my throat to prepare for the information.

As she was talking to me, she received a call from one of the laboratory hematologists who was currently looking at the sample under the microscope. I heard Dr. P explaining on the phone, "She's 29, a teacher, just had a baby..." indicating the unusual nature of someone in my age group receiving this diagnosis. But again, I had no concept or understanding about MDS. These moments for me felt like a cinematic scene playing in my mind. As she hung up the phone, she sat back down on the stool and explained what it all meant. I held my breath,



hoping it wasn't that serious as she continued, "...you'll need radiation, chemotherapy, a bone marrow transplant..." I knew all about chemotherapy. But radiation and a bone marrow transplant sounded much worse. Just hearing the word "transplant" — images of organ transplants flashed before my eyes. But a bone marrow transplant left me uneasy. Admittedly, I had never heard of it.

The tears welled, my heart pounded, the lump in my throat remained. I couldn't breathe. I looked at my mom and saw her eyes well; I knew she was trying to grasp the information. Was it cancer? How serious was this?

I was so confused. I'm sure Dr. P explained in those moments that it was blood cancer, but something about receiving a serious health diagnosis alters your ability to comprehend. Words drip out of your ears and all you can really feel is the rock forming in your gut. At the end of the visit, I walked out of the building crying with my mom's arms cradled around me. Through the parking lot you'd hear me saying through tears, "Is this cancer? Am I going to be okay? Am I going to lose my hair? What does this mean?" The reality of death was pushed to the forefront of my mind. Would this thing—that I apparently now have—be my end? What about my daughter? Would I really only be a mother for a short time? And my husband, Nate. These are the cold, hard thoughts that instantly hit you. And these are the thoughts that change you.

We got to the car and I googled the diagnosis right away because I couldn't fully hear the pronunciation through Dr. P's accent. In doing so, I saw the words "pre-leukemia"

and was struck with more confusion. I put down my phone and cried into my knees. My mom called my dad quickly and I called my husband. Nate left his work right away, and my mom and I drove home.

The remainder of the day consisted of tears, hugs, prayers, and confusion. Dr. P had given us the contact information for a couple specialists, and we managed to get an appointment with the specialist the next day. And so the journey, as they say, began.

### SO I DANCED

Sometimes you have to choose joy in life. This photo was from my sweet friend's wedding a few days after my diagnosis. I remember feeling the importance of enjoying that evening. I remember the sense of looming change in the air and I remember the feeling of wanting to savor the present—to dance, to eat, to look in to my husband's eyes, and to just simply take in the moments of life that are presented to you. I've always been the type of person that loves dancing at weddings. At this wedding in particular, it was difficult to dance with the anemia, but it was everything in those moments.

### CONNECTION AND COLLOQUY

Quickly following my diagnosis, I felt the immediate need to find other patients with MDS. However, little did I know how difficult that would be. In the months following my treatment, I contacted the MDS Foundation and discovered an incredible platform to connect with others called Colloquy. Essentially, Colloquy provides the MDS community opportunity to share personal stories and hear real-life experiences. If you are searching to connect with others, hear their journeys, or just find resources, I highly recommend you check out the platform. Additionally, in the months following my transplant, I felt the need to write about my journey in a blog called, *Whirlwind and Wonder*: <https://michellecolu.wixsite.com/whirlwindandwonder>. Every few months, I'll share the ups and downs that I have experienced throughout my recovery process. Connecting with others that have gone through a similar experience has been a huge encouragement.

## WE ARE MANY STORIES

### BECKY TOOMAY

Dallas, Texas

There are so many forms of myelodysplastic syndrome. I'll share my journey with you knowing that you understand that we are all different. I am a 74-year-old female living in Dallas, Texas. The first inkling of a problem with my blood was during an annual physical in the late 1990s. My internist mentioned that there were some enlarged red blood cells that we would need to keep an eye on. My hemoglobin was normal in 2010 at 13. It began falling each year in spite of supplements and eating chicken liver. In 2016 when the hemoglobin hit 9 my internist recommended I see a hematologist.

I was a bit surprised when the receptionist answered the phone saying Hematology/Oncology. Hmmmm. Based on my history the hemotologist recommended a bone marrow biopsy which he did that day. I returned a week later for the results. I had MDS RARS – low risk. This kind, young doctor looked me in the eye and recommended that I find a doctor that specializes in MDS.

So the research began. I found the MDS Foundation, read extensively, listened to webinars on their website, and went to one conference in person. Thank goodness for computers and the MDS Foundation. I am lucky to live in a big city with a teaching hospital, University of Texas Southwestern. UTSW is also a Center of Excellence for MDS. I saw Dr. Robert Collins one month after diagnosis in January 2017. Tissue samples from the December biopsy were sent to Foundation One for genetic sequencing. The results showed I had one mutation, SF3B1, which is considered a 'good' mutation because of its positive prognosis. For the next 4 and a half years I was 'watching and waiting' with no treatment. I had blood draws every six months.

In late May 2021 my sons and I went rafting through the Grand Canyon. I noticed significant bruising and requested a blood draw when I returned. Hemaglobin, platelets, and neutrophils counts were dropping. In August I had another bone marrow biopsy



Becky and her sons – "We Lucky 3"

with tissue again sent to Foundation One. I had two new mutations: ATRX and GNAS indicating a poorer prognosis. Knowing what my mutations are has helped direct my treatment.

At this point Dr. Yazan Madanat took over my care. He specializes in the treatment of MDS. I was having weekly blood draws. In November when my Hgb was 7, I had my first blood transfusion. In February 1922 I had my second blood transfusion.

Meanwhile the COVID pandemic was at its height. I was very careful and went only to my sons' backyards and UTSW. Groceries were ordered. I did a zoom exercise class 3 days a week and walked 2 times a week. I was lucky I was in no pain, just tired with some shortness of breath. I read, gardened, painted, watched

movies and had several friends who visited in my back yard, spaced and masked.

Then there was a dramatic decline in my platelets and Dr. Madanat discussed options. I am not interested in a stem cell transplant. I don't feel bad enough. There was a clinical trial that looked very promising but the FDA put it on hold just when I needed it. So we decided on chemotherapy in pill form, INQOVI. I had a platelet transfusion on March 3 followed the next day by another bone biopsy. I started INQOVI March 12: a pill a day for the first 3 days of a 28-day cycle. As expected my blood counts fluctuate during each cycle. I've had no side effects. I've had 2 more platelet transfusions and manage low neutrophils with antibiotics and antivirals. I had a bout of diverticulitis that required IV antibiotics in the hospital.

The weeks of triple digits this summer have burnt my garden to a crisp. No one wants to visit outside except at dawn. I continue with cycles of INQOVI. My Hgb has stayed around 9. Platelets and neutrophils go from normal to surprisingly low. At the moment we are varying the length of the cycle and the pill schedule hoping to give the bone marrow a chance to kick in and produce cells that mature. Meanwhile I eat well, sleep enough, and exercise. I feel so lucky to have at my disposal the wealth of information from the MDS Foundation. They guided me to UTSW as a Center of Excellence. Being in the capable hands of Dr. Yazan Madanat and his capable team reassure me and give me the peace of mind I need to continue on this journey.

The best to all of you.



Becky with her sisters



## EVERY DAY IS A BLESSING

### RICHARD SCHNEIDER

*Stanhope, New Jersey*

My name is Richard Schneider and I was officially diagnosed with MDS in 2013. I live in northwestern New Jersey with my wife of 54 years. That is when my first bone marrow biopsy (BMB) showed that I had MDS-RS (previously known as RARS) with the SF3B1 DNA mutation and no chromosome involvement. My actual journey actually started before this, however. As early as 2005, my red blood count and hemoglobin were below normal and my MCV was elevated. This declining trend continued for a number of years. When I discussed this with my internist he wasn't concerned. He said these counts were probably just my "normal" counts. I proceeded to chart/graph my blood counts going back to 1984 which definitely showed the decline over the years. My doctor was unavailable the day of my appointment due to a family emergency, so I showed my chart/graph to his associate. She did not offer a diagnosis but agreed that something was going on and recommended I see a hematologist. A short time later, I had my first visit to an endocrinologist who commented on my anemia and recommended I see a hematologist. Coincidentally, it was the same hematologist/ oncologist the previous doctor recommended. When the hematologist/ oncologist looked at my chart/graph he immediately suggested that it was probably low-risk MDS, and he ordered a BMB. I had never heard of this disease. While waiting for the results of the biopsy, I did some research. I read everything I could find from reliable sources including the MDS Foundation. What I learned was terrible. It is a cancer and there is no cure. According to the international scoring system the median life expectancy for patients with a low-risk form of the disease was eight years. My whole world crashed; I would be dead in eight years. I had young grandchildren that meant the world to me, and I would not be part of their lives growing up to share in their birthday parties,



graduations, and various celebrations. I was in complete disbelief.

The results of the BMB showed that I did in fact have MDS. Specifically, it was the MDS-RS subgroup (formerly RARS). There was no chromosome involvement, but I did have the SF3B1 mutation which is common for this MDS subgroup. My blast count was 2% which I later learned was a good thing. I had a good heart to heart discussion with my doctor. He explained everything and calmed me down. I should point out that he is not an MDS specialist but he is very familiar and knowledgeable about the disease. He is the smartest, most knowledgeable, and compassionate doctor I have ever had in my life. I have his email and he always gets back to me promptly if I have a question. For many years, he was the head of the cancer department at a major NJ Medical center. I spoke to him about seeing an MDS specialist at a MDS Center of Excellence which he encouraged me to do so and recommended several. He also wrote a letter of introduction for me.



*My Grandchildren*

I made an appointment with Dr. Stuart Goldberg at the John Theurer Cancer Center at Hackensack University Medical Center. We talked about my diagnosis and the disease in general for over an hour. He confirmed the diagnosis and the course of action (watch and wait) that my local oncologist was recommending. He also discussed what to expect going forward, what to watch out for and suggestions on how to deal with those issues. Unfortunately, he was cutting back on his practice, so it was not feasible to continue to see him. He recommended Dr. Azra Raza at New York Presbyterian Hospital. He did, however, give me his email and remained in contact with me until his retirement. He was a valuable resource – I miss him.

Dr. Raza performed a BMB at my first visit confirming the diagnosis. I have found her to be compassionate and approachable as well. While on watch and wait, I saw her twice a year. I saw my local oncologist every four months to have my blood counts monitored. The results were shared with Dr. Raza. My local oncologist is more convenient than going into the city for routine blood work.

After seven years on watch and wait, the disease has progressed. My red blood count and hemoglobin had decreased to the point where I needed active treatment. The BMB also showed that I had a second DNA mutation, ASXL1. I have been getting Aranesp and Neupogen injections every four weeks at the local cancer center, since then. Having blood work done every four weeks is somewhat annoying, but not overly intrusive. Everyone at the center is very nice. I feel like Norm from the TV series Cheers. Everyone knows my name and I know theirs. The injections have been keeping my hemoglobin in the 10 – 11 range.

### PHYSICAL AND EMOTIONAL IMPACT

I already mentioned my reaction when I was initially diagnosed. I was in disbelief. I still have trouble wrapping my head around it. The disease is progressive. I worry about when it will progress further and how bad it

## OUR PATIENT STORIES

may be. My doctors believe I will die with the disease and not from it. At some point the Injections may stop working which is not atypical and the treatment will have to be more aggressive. How aggressive is not known. The main impact at this point is fatigue, shortness of breath and a general lack of stamina. Fatigue is not the same as being tired. If you are tired you can take a nap. If you are fatigued and take a nap, you are still fatigued when you wake up. When doing even light yard work, I have to rest every few minutes. I appear "normal" to others, so they don't see these effects and they don't fully understand this disease, not even my family.

### THE BLESSING

Every day is a blessing. I fight my fears daily. I'm grateful that the progression, so far, has been slow. For the past nine years, I have been able to attend countless celebrations and events in my grandchildren's lives. I have



*Celebrating with our children and grandchildren is a blessing.*

been to countless soccer games, football games, basketball games, baseball games, Lacrosse games, etc. and loved every minute of it! In the next two years, I will be attending two high school graduations. My wife and I are able to go on vacations, attend shows, and go out to dinner frequently. All of this is what I live for. I do not know what the future will bring, but I'm trying to enjoy the here and now!



The mission of Angel Flight East is to provide free air transportation to qualified patients and their families by arranging flights to distant medical facilities, delivering supplies to disaster areas, and reuniting families during desperate times.



[angelflighteast.org](http://angelflighteast.org) - 215-358-1900



## FROM CAREGIVER TO PATIENT: BEAUTIFUL IRONY

**LINDA MARTENSEN**  
Englewood, Florida

In 2011, my husband Rusty and I sold our home in NJ and moved to FL as part of the “retirement” plan to enjoy our families, the beautiful climate, plentiful golf courses, and lovely beaches. The most critical part of our plan, was to be near a major national cancer center, as Rusty’s myelofibrosis was clearly worsening.

Myelofibrosis is a rare, chronic blood cancer. People with MF, have a defect in the bone marrow, that results in the abnormal production of blood cells, causing scar tissue to form. Rusty was diagnosed with Stage 4 myelofibrosis, with very high odds of progression to Acute Myeloid Leukemia. Much of our life revolved around long commutes to northern NJ/NY cancer centers for consultations, bone marrow biopsies, various procedures, chemotherapies, and transfusions. At that time, very little was known about myelofibrosis and we were fortunate that we were able to see some of the top people at Cornell. The long travel distances, the caregiving, and trying to maintain our professions, was taking its toll. We felt our quality of life would improve by living closer to a major cancer center and being physically closer to my adult children, who lived in FL. We believed if Rusty were able to get a bone marrow transplant, we would have family nearby to help us through everything. So, we were off to Tampa.

Our “vision” came to an abrupt halt when after 48 hours in FL, Rusty was admitted to the Moffitt Cancer Center. While our cars were being shipped, our belongings on a moving van, and our closing date on our new home scheduled, Rusty was being diagnosed with Acute Megakaryoblastic Leukemia (M-7). This subtype is considered rare with a very poor prognosis, especially since it was not de novo. After much consultation, testing, procedures and discussion of the risks, we decided to try the pre transplant process, even



though there was no possibility of a match. After 28 days in the hospital, my husband was given 2 months to live. He lived for 4 months due to his bravery, heroism and strong spirituality. During Rusty’s time in the hospital, I tried to put the pieces of our move together, closing on the house, moving us in and bringing some order to all that had happened. Daily hospital visits, were of course, the top priority.

When Rusty came home, I was proud of all that we had managed to do but heartbroken at the prognosis. I lost him and lost myself in the grief and mourning that followed his death. Nothing about his death made sense. He was “supposed” to be here; he was only 52 years old.



My husband, Rusty

I was exhausted from the years of caregiving, the interstate move with all of its many complications, the intense emotional and psychological issues during the pre/post transplant process, and the deep sadness that enveloped me.

Never did I think, however, or would I have believed, that I would be a cancer patient myself, only three months after my husband’s death. Yes, *not only a cancer patient but one with a rare blood cancer.* Ironic? Absolutely. Surreal? Definitely. It was a double whammy, complete with flashbacks of the chronology of Rusty’s life and the path his disease had taken. It seemed to be an impossible irony.

NEVER DID I THINK, HOWEVER,  
OR WOULD I HAVE BELIEVED,  
THAT I WOULD BE A CANCER  
PATIENT MYSELF, ONLY  
THREE MONTHS AFTER MY  
HUSBAND’S DEATH.

My own cancer journey began as it did for many of you: with routine bloodwork and a very unexpected outcome.

My annual check up, complete with the requisite CBC, was scheduled a month after Rusty’s death. I was at ground zero after my husband’s death: not eating or sleeping and plagued by chronic fatigue. I lost 17 pounds which I attributed it to all that had happened.



## OUR PATIENT STORIES

But I was still shocked by the extremely low hemoglobin, coupled with the unusual white blood cell and platelet counts. Since these “numbers” were clearly very familiar to me, I began to be quite anxious. The physician began the immediate implementation of all the procedures I knew only too well: further examination of ferritin, analysis of B-12 results and a range of tests to discover the root cause of the anemia. I felt panicky because I knew where this was heading as the physician referred me to a hematologist oncologist. I left the office never intending to act on the referral. I vowed to eat better, sleep better and improve my “scores.” In my mind, it was simply impossible that this could be happening to me. In complete denial, I reasoned that the stress had made me tired, the FL heat had caused breathlessness and the bruises were a result of moving all that furniture. My daughter, however, insisted that I see the specialist. Another irony, my daughter worked in the pharmaceutical industry in the area of malignant hematology. Although firmly in denial, I complied with the colonoscopy, endoscopy, “pill cam” review of my lower intestine, and more bloodwork. I secretly hoped for any cancer but a blood cancer.

The following month at the big “reveal” appointment, my denial was totally shaken as the need for a bone marrow biopsy was clear. Those three words: “Bone marrow biopsy” are loaded with emotion for me. They conjure up the day that Rusty’s cancer journey began and all that followed. Since I now needed the bone marrow biopsy, I now had to return to Moffitt as the cancer patient. This was possibly the worst part of it: that I had to go to the same hospital that my husband had been in, the same valet parking lot, the same blood draw rooms, the same doctors, the same clinic where we had spent countless hours... again, ironically, the same well reputed cancer center that I had sought out.

The biopsy did confirm a MDS diagnosis. I had to believe it even though it felt like a cruel joke. In hindsight, I was blessed to have had a diagnosis that went smoothly and an early identification of my cancer.

My particular subtype is MDS-RS-SLD with a low IPSS score. A watch and wait protocol was in effect for several years which gave me time to process my cancer diagnosis and the opportunity to learn about my disease. Now, I can more rationally differentiate my spouse’s disease from my own. I understand from the data: mutation, blasts, counts etc. that my odds of getting AML are low and that my prognosis is certainly not Rusty’s. It took me a long time to get to this point. In 2021, I began weekly treatments since my hemoglobin was trending lower. My care has been excellent with a high level of coordination between Moffitt Cancer Center and FL Cancer Specialists. Beginning treatment was another hurdle for me emotionally as my cancer journey became more “real”. Chronic fatigue is always present and fighting through it is sometimes a struggle. I have a lot of joint and muscle pain that has forced restriction in my activities. Last month I was evaluated for a possible autoimmune disease which included another round of tests and procedures. Results were negative so the swelling and pain were attributed to either the inflammatory component of MDS or the side effects of my treatment. I find acupuncture, yoga, deep breathing techniques and strategies for positive self talk, the most helpful in dealing with the emotional and psychological impact of cancer. At the same time, nothing replaces good friends and family with whom I can share my true feelings. I am grateful for them. They are the core topic of the “gratitude” walks that I take daily.

I often reflect upon the *irony* of having transitioned from caregiver to patient in what felt like a heartbeat. To me, caregiving, although exhausting, demanding and sometimes frustrating, is one of the best jobs you can have. You get to spend a lot of truly intimate time with your loved one. You get a rare chance to give the person you love unconditional support. Caregiving requires patience, energy, and empathy as you deal with all the aspects of the patient’s disease as well as your own reactions to it. Sometimes when your heart is breaking, at all your loved

one is going through, you continue to be upbeat and positive to help him/her get through it, shielding and protecting. The role includes managing and going to appointments, interpreting what the physician/nurse is saying, giving medications, following treatment plans, making healthy meals, working out insurance and financial issues and dealing with unexpected emergencies. It is exhausting! And so complex, as you navigate reality with potential loss. Caregiving certainly forces you to examine your own mortality as well as that of the person you love. Both cancer patient and caregiver are fatigued in the care and management of the disease.

As the cancer patient, you face all of same issues as the caregiver: needing to be patient, gathering up energy, facing the full gamut of emotions, your legacy, your priorities. Sometimes you have to be upbeat and positive for the sake of the caregiver. Shielding and protecting is not one sided.

“One size does not fit all” in caregiving and it takes effort to come to a happy, safe place for both parties. Sometimes I was super proud of my efforts and some days I felt I had really failed. I often feel that way as the patient.

The ironic silver lining of cancer, whatever your role, is that it forces you to look more deeply and clearly at your life:

***Beautiful irony is when the very thing that tried to destroy you, instead made you stronger.***

I really believed that caring for a loved one who passed from cancer and then getting diagnosed with cancer myself would break me. But I realize that it made me stronger in many ways. I am more accepting of others and the problems they may be having that are unknown to me. I try to be kinder and more empathetic.

“I’m still standing” so I guess I am learning to cope and turn a lot of my anxiety and fear around. I try very hard to focus on self talk that is less negative and distorted. I think I have always been resilient but cancer tests you like no other. In a way, the cancer diagnosis, for me, represents one of the longest, continuous

## OUR PATIENT STORIES

growth cycles in my life. Important priorities no longer include “stuff”: material things have taken a back seat to trying to spend time with family and close friends. I am more grateful and appreciative of all that I do have. Without my wonderful family and many dear friends I would not be doing as well as I am. Their support has meant everything.

Still, one of my major challenges is acknowledging that I cannot do a lot of what I did before. I just do not have the stamina and energy that I once had. I have a very hard time saying that aloud; it is growth that I am actually sharing it now! I am very independent and asking others for help or admitting that I cannot do something is very foreign to me. I am learning that my loved ones want to help me if I will only let them. I realize from the caregiving experience that helping people you love brings incomparable joy.

Another big challenge for me is to face the fact that MDS, at this point, is treatable but not curable. Sometimes the fact that there is no clear path to a cure overwhelms me.



Linda and Jack

However, I need only to look at my own experience to feel hope. When Rusty was diagnosed with myelofibrosis, bone marrow transplants for this condition were considered “experimental”. The research on myelofibrosis has come a long way. It has and will for MDS as well. I am so encouraged by the

research and resources provided by the MDS Foundation. Because of the Foundation’s educational initiatives, research and efforts to create patient advocacy groups, I am in a much better place.

A final beautiful irony: sometimes “detours” in our life lead us to people and places that we could not have imagined. I met Jack several years after Rusty passed. Jack also lost his beloved to cancer. We now share a happy life together. Jack is a wonderful partner and a kind and loving caregiver. He is the “zen” to my type A personality and helps me in so many ways to manage my cancer. I like to think Jack was sent to me by my angel, Rusty, who knew the kind of love and support I would need on this challenging journey.

Special thanks to Audrey Hassan for encouraging me to share my story with you. As I read the unique stories of my fellow cancer survivors, I feel humbled and inspired by your courage. Your bravery helps me to feel connected and energized to move forward. Thank you.



Give  
IN 2022

**YOU CAN BE  
SOMEONE'S**  
*new year*

Let's change the future of MDS.  
Put the Foundation on your gift  
list this holiday season.

**mds foundation**  
the myelodysplastic syndromes foundation, inc.





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

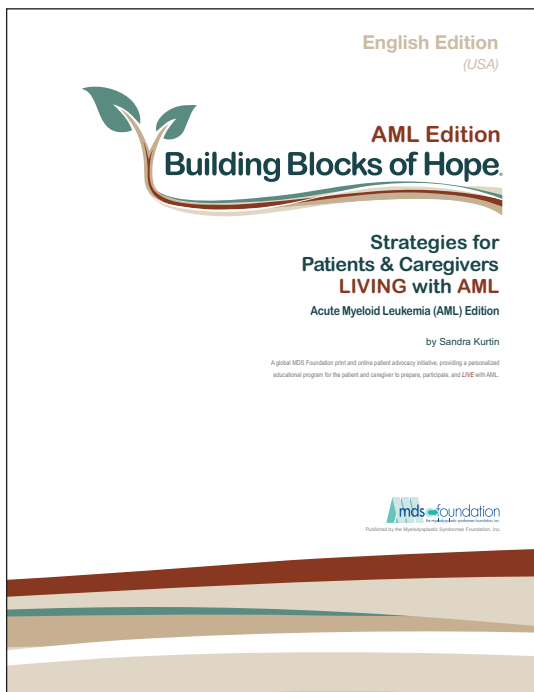
This resource is intended for patients with acute myeloid leukemia (AML). You will find expert advice about AML, AML with myelodysplasia-related changes (AML-MRC) and treatment-related AML (tAML) to help you discuss key issues with your healthcare provider and make important decisions related to management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

### “YOU AND AML” CONTAINS 4 LEARNING MODULES:

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

### “YOU AND AML” – NEW MODULES AVAILABLE!

- Maintenance and Continuous Treatment in Acute Myeloid Leukemia
- Treatment Failure and Relapse in Acute Myeloid Leukemia



## BUILDING BLOCKS OF HOPE

*You or someone you know has been diagnosed with AML.*

Hearing the words Acute Myeloid Leukemia or AML can be frightening. The diagnosis of AML is often unexpected and filled with both immediate and long-term challenges. You probably have many questions. Allow yourself time to adjust to the diagnosis of AML. Take time to explore the Building Blocks of Hope®, it is designed to help get you the information that you are looking for and take an active part in your AML journey. This is a great way to share this information with family and friends. The AML BBoH contains four chapters and a glossary of terms:

**Chapter 1:** Understanding Acute Myeloid Leukemia

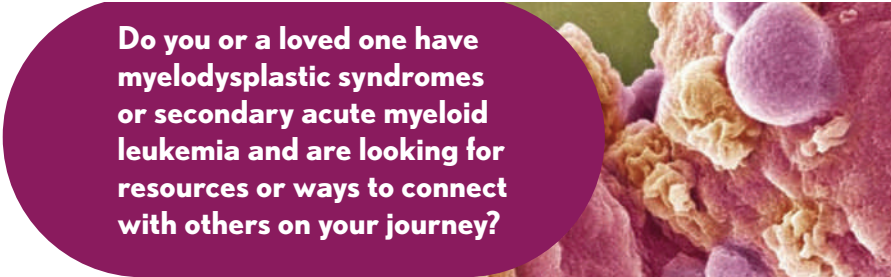
**Chapter 2:** Seeking Treatment

**Chapter 3:** General Resources for Living with AML

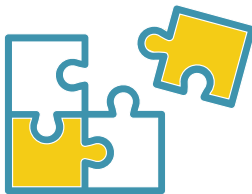
**Chapter 4:** The MDS Foundation

# FIND THE RIGHT FIT

Navigating Secondary Acute Myeloid Leukemia

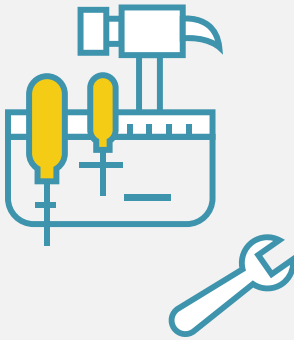


Do you or a loved one have myelodysplastic syndromes or secondary acute myeloid leukemia and are looking for resources or ways to connect with others on your journey?



People affected by myelodysplastic syndromes (MDS) or secondary acute myeloid leukemia (sAML) often face many uncertainties on how these diseases develop and progress, what available treatment options there are and the impact they may have on everyday life.

For those in search of answers, Find the Right Fit can provide information and educational resources for people living with MDS or sAML, as well as their loved ones who often take on the role of caregiver.



A cancer diagnosis can be overwhelming, but **knowledge is power**. To help navigate each person's individual journey, Find the Right Fit **provides a collection of tools** including articles, videos, patient stories and more that:



Educate on the science behind MDS and sAML



Offer information regarding treatment options and coping strategies



Connect patients and their loved ones with the appropriate resources to manage an MDS or sAML diagnosis with confidence

Visit [FindTheRightFit-sAML.com](http://FindTheRightFit-sAML.com).

## About AML



▶ AML is an aggressive (fast-growing) disease in which too many myeloblasts (immature white blood cells) are found in the bone marrow and blood.<sup>1,2</sup> sAML is one type of AML that may be linked to specific preexisting conditions, like MDS, or to prior treatment for a malignant or non-malignant disease.<sup>3</sup>

Visit [FindTheRightFit-sAML.com](http://FindTheRightFit-sAML.com) to learn more about sAML subtypes and treatment options.

## About MDS



▶ MDS, a form of blood cancer, are an often unrecognized, under-diagnosed, rare group of bone marrow failure disorders where the body can no longer make enough healthy, normal blood cells in the bone marrow.<sup>4</sup>

▶ The cause of MDS is unknown, but potential triggers include radiation and chemotherapy for cancer, as well as long-term exposure to certain environmental or industrial chemicals, such as benzene.<sup>4</sup>

Visit [FindTheRightFit-sAML.com](http://FindTheRightFit-sAML.com) to learn how MDS can progress to sAML.

Find the Right Fit is a program from **Jazz Pharmaceuticals**, developed with consultation from the **Myelodysplastic Syndromes Foundation, Inc.** and the **Cancer Support Community**.



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## IT'S OKTOBERFEST TIME! EIN PROSIT!

**SHIRLEY O'BRIEN**

*Tucson, Arizona*

Although we sing and accompany ourselves with our accordions all year round, October is the best month because we perform those well-known favorite songs such as Beer Barrell Polka, Happy Wanderer, Edelweiss and so many more. We have performed on many Oktoberfest celebration stages over the last few years but as I write this, I vividly remember last night's two-hour performance for a couple of hundred enthusiastic residents of a large Senior Retirement Village. They were ready to party with our music and an awesome authentic German dinner accompanied by a good amount of German beer. We taught the "Chicken Dance" when everyone was enjoying more after-dinner liquid refreshments. They loved it.

When I was diagnosed with MDS in February 2012, I thought our time performing at Oktoberfest celebrations was over. At the time, my spouse, Jim, and I had been retired for eight years as professors at the University of Arizona and we enjoyed traveling in our motorhome to summer festivals.

We had planned a cruise in less than a month and my oncologist encouraged us to go, saying that once I got into treatment our lives would be significantly different. However, we decided to cancel the cruise and begin treatment immediately. I had a port catheter implanted and the treatment began.



When summer rolled around, we made a firm decision: we would keep our music going as long as possible. We continued to rehearse to be ready. There is nothing like music to elevate the mood and encourage the soul. We accepted invitations to Scandinavian, Italian, and German festivals at and somehow were able to fit those festivals around my treatments of 7 days every 28 days. The ethnic music and the audience's appreciation carried me along enough to perform, perhaps not my A-game, but enough to be asked back another year.

After three and a half years of this treatment regimen, my blood counts continued to steadily slide lower. They cut the dose in half and then stopped treatment altogether, telling me it was no longer effective. It had failed. After another bone marrow biopsy with a biomarker test, a genetic mutation (the IDH1 gene) was identified. It was after that test that an oncologist said: "Shirley, I'm sorry but you have five to seven months to live." I wasn't willing to accept this so I sought a second opinion and got an even more grim prognosis but this was accompanied by "Shirley, you need to find a clinical trial and soon."

So, I got to work. As a retired professor, I took this on as a research project that was very, very personal. For days, I searched [clinicaltrials.gov](http://clinicaltrials.gov) without success. I needed

help and an MDS patient counselor found a trial with a focus on my specific mutation. It was a Phase II clinical trial at MD Anderson Cancer Center, using an experimental drug called Ivosidenib/Tibsovo which is a targeted anti-cancer medication for the treatment of acute myeloid leukemia.

A few days after I applied to become a new patient at MD Anderson, Dr. Courtney DiNardo asked me to fly from my home in Tucson for testing. Between MD Anderson's huge campus and the battery of medical tests, our first visit was overwhelming, but they gave me hope that this clinical trial just might be the right one for me.

Only 24 hours after my spouse and I returned to Tucson, Dr. DiNardo called and said she had reviewed my test results and asked us to return right away. So, we canceled our holiday plans, packed our belongings, and arrived in Houston on Dec. 12, 2015.

At MD Anderson, we learned that my MDS had progressed to acute myeloid leukemia (AML). That was a shock, but at the same time, we felt we were right where we needed to be.

I was accepted into the trial and on December 23, 2015, and I took my first dosage of Tibsovo. At that moment I took a selfie with the two techs because I had this strong sense that this was going to be my game changer. Then came endless EKGs, blood tests, and bone marrow biopsies to check my blood cell counts. Two weeks into the clinical trial, my white cell count was higher than it had been in two years. We were amazed, elated and hopeful.

When I arrived at MD Anderson, my blast count was at 30%, well into the AML range, which begins at around 20%. At the end of the first 28-day cycle, it was just 2%, which is normal. My blast count was the first part of this miraculous experience.

My blood count values -- red blood cells, white blood cells, and platelets -- reached near normal range within six months and low-end normal in a year. Whenever Dr. DiNardo's team asked about side effects, I



## OUR AML PATIENT STORIES

could not name any except I sing better and play more exciting accordion music.

I'll always remember what Dr. DiNardo said the day before I was accepted into the clinical trial: "You are in the right place at the right time with the right mutation." And, she was absolutely right.

When you are diagnosed with a disease like MDS and AML, it is important to research it and become your own advocate because no one else is going to do it for you. However, I had excellent support from my spouse, Jim, who has been with me at every step of this journey. When Dr. DiNardo said we needed to travel to Houston for treatment for two or three months, I just couldn't wrap my mind around how we could do it. Jim immediately said: "Of course, we will come, we have a motor coach. We will be there in a few days". And we were. I could never have gotten through this without his constant positive encouragement. He has been my rock and my accordion duet partner.

Finally, our faith and the prayers of so many of our family and friends have provided and continue to provide consolation, confidence, and cheer. I am willing to tell my story to anyone who will listen. My story is a story about a miracle. I am willing to talk with



I AM WILLING TO TALK WITH ANYONE WHO IS BEGINNING THIS JOURNEY OR WHO IS DISCOURAGED AND NEEDS TO KNOW THERE ARE NEW CANCER TREATMENTS DEVELOPED EVERY DAY AND THERE IS HOPE AND HELP AVAILABLE.

anyone who is beginning this journey or who is discouraged and needs to know there are new cancer treatments developed every day and there is hope and help available.

I have now been in remission for the past 10 years and have enjoyed a full life of musical performances such as last night.

This reminds me of a conversation as we were leaving our Oktoberfest performance last night, "Shirley, my friend's husband has just been diagnosed with leukemia and they are so scared. Will you talk with her?"

Of course.

## DID YOU KNOW?

**The Myelodysplastic Syndromes (MDS) Foundation, Inc.** was established by an international group of physicians and researchers to provide an ongoing exchange of information relating to MDS.

Until the Foundation was set up, no formal working group had been devoted to MDS. Since its inception, we have conducted 16 international symposia in Austria, England, the United States (Chicago, Washington, DC), Spain (Barcelona, Valencia), Czech Republic, Sweden, France, Japan, Italy, Greece, Scotland, Germany, Denmark, and Canada. We are looking forward to our 17th International Congress in Marseille, France on May 3-6, 2023.

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

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

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

Learn more about  
**The Myelodysplastic Syndromes Foundation, Inc.**  
and find additional resources here:  
[www.mds-foundation.org](http://www.mds-foundation.org)



## SERVIER ANNOUNCES FDA APPROVAL OF TIBSOVO® (IVOSIDENIB TABLETS) IN COMBINATION WITH AZACITIDINE FOR PATIENTS WITH NEWLY DIAGNOSED IDH1-MUTATED ACUTE MYELOID LEUKEMIA

- TIBSOVO is the first therapy targeting cancer metabolism approved in combination with azacitidine for patients with newly diagnosed IDH1-mutated acute myeloid leukemia
- FDA approval based on data from the global, Phase 3 AGILE trial that demonstrated a statistically significant improvement in event-free survival and overall survival

**BOSTON, MASS. MAY 25, 2022** – Servier, a leader in oncology committed to bringing the promise of tomorrow to the patients we serve, today announced that the U.S. Food and Drug Administration (FDA) approved TIBSOVO® (ivosidenib tablets) in combination with azacitidine for the treatment of patients with newly diagnosed IDH1-mutated acute myeloid leukemia (AML) in adults 75 years or older, or who have comorbidities that preclude use of intensive induction chemotherapy. TIBSOVO is the first therapy targeting cancer metabolism approved in combination with azacitidine for patients with newly diagnosed IDH1-mutated AML. The AGILE trial was the only Phase 3 trial designed specifically for newly diagnosed patients with IDH1-mutated AML who are ineligible for intensive chemotherapy.

The supplemental New Drug Application (sNDA) for TIBSOVO received Priority Review and was reviewed by the FDA under its Real-Time Oncology Review (RTOR) pilot program, which aims to ensure that safe and effective treatments are available to patients as early as possible.

“Today’s approval builds on the established body of evidence for TIBSOVO,

which is now approved across multiple IDH1-mutated cancer types,” said David K. Lee, Chief Executive Officer, Servier Pharmaceuticals. “As a leader in oncology pioneering the science behind targeted IDH inhibition, we are proud to bring a new therapeutic option to the acute myeloid leukemia community and remain committed to pushing the boundaries of health-care innovation in oncology and beyond.”

IN ADDITION TO A FAVORABLE SAFETY PROFILE, TIBSOVO IS THE FIRST THERAPY TARGETING CANCER METABOLISM TO DEMONSTRATE AN IMPRESSIVE, SIGNIFICANT BENEFIT IN EVENT-FREE SURVIVAL AND OVERALL SURVIVAL IN COMBINATION WITH AZACITIDINE...

The expanded approval of TIBSOVO is supported by data from the AGILE study, a global, Phase 3 trial in patients with previously untreated IDH1-mutated AML. Results from the AGILE trial demonstrated a statistically significant improvement in event-free survival (EFS) (hazard ratio [HR] = 0.35 [95% CI 0.17, 0.72], 2-sided p-value = 0.0038) and overall survival (OS) (HR = 0.44 [95% CI 0.27, 0.73]; 2-sided p = 0.0010). TIBSOVO plus azacitidine treatment resulted in a threefold improvement in median OS (24 months) compared to placebo plus azacitidine (7.9 months) as a first-line treatment for IDH1-mutated AML. Results from the AGILE study were presented at the 2021 American Society of Hematology (ASH) Annual Meeting and Exposition, and recently published in the *New England Journal of Medicine* (NEJM).

“Acute myeloid leukemia is a rapidly progressing, difficult-to-treat blood cancer with a poor prognosis,” said Eytan M. Stein, M.D.,

Director, Program for Drug Development in Leukemia, Leukemia Service, Department of Medicine at Memorial Sloan Kettering Cancer Center. “In addition to a favorable safety profile, TIBSOVO is the first therapy targeting cancer metabolism to demonstrate an impressive, significant benefit in event-free survival and overall survival in combination with azacitidine, underscoring its importance as part of a new combination regimen for patients with newly diagnosed IDH1-mutated acute myeloid leukemia who are not candidates for intensive induction chemotherapy.”

AML is a difficult-to-treat cancer of the blood and bone marrow and is one of the most common types of leukemia in adults with approximately 20,000 new cases estimated in the U.S. each year. IDH1 mutations are present in about 6 to 10 percent of AML cases.

“People living with acute myeloid leukemia, especially those who are newly diagnosed and are not eligible for intensive chemotherapy, have had few treatment options,” said Susan Pandya, M.D., Vice President Clinical Development and Head of Cancer Metabolism Global Development Oncology & Immuno-Oncology, Servier. “Today’s approval of TIBSOVO in combination with azacitidine represents a major advancement for patients with newly diagnosed IDH1-mutated acute myeloid leukemia in the United States, and we look forward to continuing our engagement with regulatory authorities worldwide.”

The combination of TIBSOVO plus azacitidine demonstrated a safety profile consistent with previously published data. The most common adverse reactions (≥10%) in newly diagnosed AML patients receiving TIBSOVO in combination with azacitidine were nausea, vomiting, electrocardiogram QT prolonged, insomnia, differentiation syndrome, leukocytosis, hematoma, hypertension, arthralgia, dyspnea, and headache. The select laboratory abnormalities (≥10%) were leukocytes decreased, platelets decreased, hemoglobin decreased, neutrophils decreased, lymphocytes increased, glucose increased, phosphate decreased, aspartate

aminotransferase increased, magnesium decreased, alkaline phosphatase increased, and potassium increased.

The recommended dosage of TIBSOVO for newly diagnosed IDH1-mutated AML is 500mg once daily via oral administration.

In an effort to support the patient communities it serves, Servier Pharmaceuticals recently introduced ServierONE Patient Support Services, a program that offers one-on-one support to help patients who are prescribed TIBSOVO or other Servier products navigate their cancer journey. Eligible patients will have access to financial assistance, emotional support and other resources. More information can be found at [www.servierone.com](http://www.servierone.com).

TIBSOVO[i] is also approved in the U.S. as monotherapy for the treatment of adults with IDH1-mutant relapsed or refractory AML, and for adults with newly diagnosed IDH1-mutated AML who are ≥75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy. Last year, TIBSOVO garnered its first approval in a non-hematologic malignancy for patients with previously treated IDH1-mutated cholangio-carcinoma.

### ABOUT THE NCT03173248 AGILE PHASE 3 AML TRIAL

The AGILE trial is a global, Phase 3, multicenter, double-blind, randomized, placebo-controlled clinical trial designed to evaluate the efficacy and safety of TIBSOVO in combination with azacitidine compared with placebo in combination with azacitidine, in adults with previously untreated IDH1-mutated acute myeloid leukemia (AML) who are not candidates for intensive chemotherapy (≥75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy). The study's primary endpoint is event-free survival (EFS), defined as the time from randomization until treatment failure, relapse from remission, or death from any cause, whichever occurs first. Treatment failure is defined as failure to achieve complete remission (CR) by Week 24.

Key secondary endpoints included CR rate, defined as the proportion of participants who achieve a CR; overall survival (OS), defined as the time from date of randomization to the date of death due to any cause; CR and complete remission with partial hematologic recovery (CRh) rate, defined as the proportion of participants who achieve a CR or CRh; and objective response rate (ORR), defined as the rate of CR, CR with incomplete hematologic recovery (CRi) (including CR with incomplete platelet recovery [CRp]), partial remission (PR), and morphologic leukemia-free state (MLFS).

### ABOUT ACUTE MYELOID LEUKEMIA

Acute myeloid leukemia (AML) a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults, with approximately 20,000 new cases in the U.S., and 43,000 cases in Europe each year. AML incidence significantly increases with age, and the median age of diagnosis is 68.2. The five-year survival rate is approximately 29.5%. For 6 to 10 percent of AML patients, the mutated IDH1 enzyme blocks normal blood stem cell differentiation, contributing to the genesis of acute leukemia.

### ABOUT SERVIER PHARMACEUTICALS

Servier Pharmaceuticals LLC is a commercial-stage company with a passion for innovation and improving the lives of patients, their families and caregivers. As a privately held company, Servier has the unique freedom to devote all of its time and energy towards patients who require our treatments, care and innovation in areas of unmet medical need.

As a leader in oncology, Servier is committed to finding solutions that will address today's challenges. The company's oncology portfolio includes innovative medicines designed to bring more life-saving treatments to a greater number of patients, across the entire spectrum of disease and in a variety of tumor types. Servier has significantly accelerated its investment in hard-to-treat cancers with more

than 50% of research and development dedicated to delivering significant advances in areas of high unmet need that may truly move the needle for our patients.

Servier believes co-creation is fundamental to driving innovation and is actively building alliances, acquisitions, licensing deals and partnerships that bring solutions and accelerate access to therapies.

With the company's commercial expertise, global reach, scientific expertise and commitment to clinical excellence, Servier Pharmaceuticals is dedicated to bringing the promise of tomorrow to the patients that we serve.

More information: [www.servier.us](http://www.servier.us)

### ABOUT SERVIER GROUP

Servier is a global pharmaceutical group governed by a Foundation. With a strong international presence in 150 countries and a total revenue of 4.7 billion euros in 2021, Servier employs 21,800 people worldwide. Servier is an independent group that invests over 20% of its brand-name revenue in Research and Development every year. To accelerate therapeutic innovation for the benefit of patients, the Group is committed to open and collaborative innovation with academic partners, pharmaceutical groups, and biotech companies. It also integrates the patient's voice at the heart of its activities.

A leader in cardiology, the ambition of the Servier Group is to become a renowned and innovative player in oncology. Its growth is based on a sustained commitment to cardiovascular and metabolic diseases, oncology, neuroscience and immunoinflammatory diseases. To promote access to healthcare for all, the Servier Group also offers a range of quality generic drugs covering most pathologies.

# Do you...

# KNOW AML

## ACUTE MYELOID LEUKEMIA

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

### KNOW AML

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

**For more information, visit:**

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



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## AML-MRC

There are a number of different subtypes of AML and knowing which subtype a patient has can help inform treatment and care decisions. A certain subtype called AML with myelodysplasia-related changes (AML-MRC) is diagnosed when at least 20% of a patient's blood or bone marrow is made up of immature white blood cells, known as myeloblasts, and when the patient meets any of the following criteria:

- History of a myelodysplastic syndrome (MDS) or myelodysplastic/myeloproliferative neoplasm (MPN)
- Specific changes in their DNA
- An abnormal appearance of two or more types of blood cell under a microscope

Symptoms of AML-MRC and general AML can be similar and a healthcare professional will need to carry out specific blood and bone marrow tests to make a diagnosis. AML-MRC is more common in older patients, for whom intensive chemotherapy is not usually advised and treatment rarely achieves remission.

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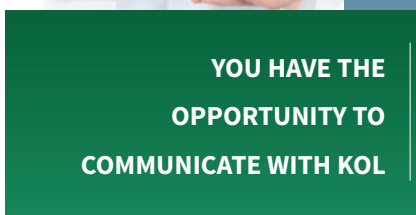
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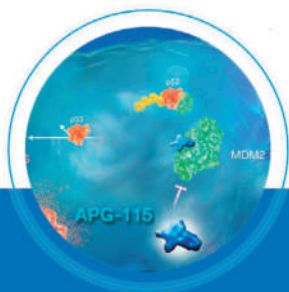
The SYROS SELECT trials are investigating **tamibarotene, a selective and potent oral RAR $\alpha$  agonist,** in certain patients with **HR-MDS** and **AML**



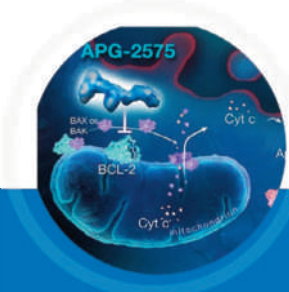
**SCAN FOR TRIAL DETAILS & INQUIRIES**

AML, acute myeloid leukemia; HR-MDS, higher-risk myelodysplastic syndrome; RAR $\alpha$ , retinoic acid receptor alpha. Tamibarotene is an investigational agent and is not approved for use in any indication in the U.S. or Europe.

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**CLINICAL TRIALS**  
**NOW ENROLLING**



**A Study of APG-115 Alone or Combined With Azacitidine in Patients With AML, CMML, or MDS**

- APG-115 is a potent, orally bioavailable MDM2 inhibitor which restores p53 expression and activates p53-mediated cell death in tumor cells.
- Recruiting patients with a diagnosis of relapsed or refractory acute myeloid leukemia (AML), chronic myelomonocytic leukemia (CMML), or high-risk myelodysplastic syndrome (MDS).
- For more information, visit <https://www.clinicaltrials.gov/> and search: NCT04358393

**A Study of APG-2575 in Combination With Azacitidine in Patients With Acute Myeloid Leukemia (AML)**

- APG-2575 is an orally active, highly selective small molecule antagonist of Bcl-2 leading to induction of tumor cell apoptosis and eventual death.
- Recruiting patients with a diagnosis of relapsed or refractory acute myeloid leukemia (AML), mixed phenotype acute leukemia (MPAL), chronic myelomonocytic leukemia (CMML), or high-risk myelodysplastic syndrome (MDS).
- For more information, visit <https://www.clinicaltrials.gov/> and search: NCT04964518



For additional questions, email: [clinical-trials@ascentage.com](mailto:clinical-trials@ascentage.com)



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The accurate completion of reimbursement- or coverage-related documentation is the responsibility of the healthcare provider and patient. Bristol Myers Squibb and its agents make no guarantee regarding coverage or reimbursement for any service or item.

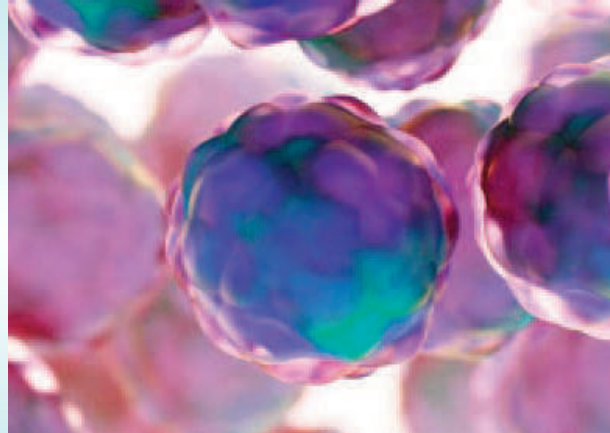
## IVOSIDENIB (AG-120)

# NEW CLINICAL TRIAL NOW ENROLLING

### Do You Have MDS with an IDH1 Mutation?

If you have **relapsed/refractory MDS** with an **isocitrate dehydrogenase-1 (IDH1) mutation**, you may be eligible for this trial.

For more information regarding this study, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT03503409).



### FOR ADDITIONAL INFORMATION ON THE TRIAL OR FOR SITES:



Please contact Servier Medical Information:  
E-mail: [medinfoUS@servier.com](mailto:medinfoUS@servier.com)  
Phone: 1-800-807-6124.

## Now Enrolling: Encore-MDS Study for Lower Risk MDS

A clinical study to evaluate the safety and efficacy of an investigational oral medication (RVT-2001) in patients with low to intermediate risk MDS who are transfusion-dependent

Consider asking if you may be eligible to take part in this study if you have low to intermediate risk MDS with an SF3B1 mutation and require blood transfusions

Participants must meet at least the following key criteria:

- Diagnosed with low to intermediate risk MDS
- Carries a SF3B1 mutation – *You may qualify if you have MDS with ring sideroblasts, but don't know your mutation status*
- Transfusion dependent
- No prior lenalidomide or hypomethylating agents (HMA) such as azacytidine or decitabine
- Failed or refractory to erythropoiesis stimulating agents (ESA)
- Be able to give written consent to participate in this trial

If you are interested in participating in this trial, please talk to your doctor about whether this study may be right for you.

For more information, email [clinicaltrials@hemavant.com](mailto:clinicaltrials@hemavant.com) or call (347) 708-7958 or visit [ClinicalTrials.gov](http://ClinicalTrials.gov) and search NCT02841540

RVT-2001 is an investigational drug that is not approved by the FDA or any other global health authority for any indication. Its safety and effectiveness have not been established.

**Advancing Research & Patient Care**

**THE 17<sup>TH</sup> INTERNATIONAL CONGRESS ON  
MYELODYSPLASTIC  
SYNDROMES**

Marseille, France | 3-6 May 2023

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