#### **SPRING/SUMMER** 2022



#### **MDS** NEWS HIGHLIGHTS

#### FROM THE GUEST EDITOR'S DESK

 THE 16TH INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES: AN OVERVIEW

Presented by: Dr. Rena Buckstein, MD, FRCPC

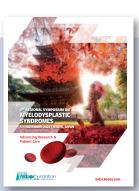
Odette Cancer Centre; Sunnybrook Health Sciences Centre
Toronto, ON, Canada



#### **PLAN TO ATTEND**

#### 3RD REGIONAL SYMPOSIUM ON MYELODYSPLASTIC SYNDROMES

**4–5 November 2022** Kyoto, Japan





## ASH 2022: MDS FOUNDATION VIRTUAL SYMPOSIUM

December 9, 2022, New Orleans, Louisiana

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www.mds-foundation.org



#### FROM THE GUEST EDITOR'S DESK

#### **GUEST EDITORIAL**

## THE 16TH INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES: AN OVERVIEW



**DR. RENA BUCKSTEIN, MD, FRCPC**Odette Cancer Centre
Sunnybrook Health Sciences Centre
Toronto, ON, Canada

The 16th International Congress on Myelodysplastic Syndromes was hosted from Toronto virtually on September 23-26, 2021 and co-chaired by Drs. Rena Buckstein and Karen Yee. 668 delegates from 44 countries spanning the globe participated. International experts both chaired and provided state of the art talks that spanned molecular genetics, immunology, prognosis, and therapeutics.

The overall objectives of the meeting were:

- Address individual needs in compliance with the Continuous Professional Development (CPD) plan.
- Identify future treatment and symptom management in patients with MDS.
- Discuss clinical experience with immune check point inhibitors in MDS.
- Describe MDS stem cell biology and therapeutic targeting.
- Discuss recent clinical and basic research with potential to improve patient care and outcomes.
- Discuss and strengthen the relevant role of nurses in the care of MDS patients.
- Integrate new knowledge of stem cell renewal and stem cell biology into the management of MDS patients.

Before the opening ceremony on Sept 23, delegates could join one of 3 concurrent sessions that included 1. workshops in morphology, flow cytometry and mouse models of MDS, 2. trainee advice sessions

that included genomics for clinical diagnostics in myeloid neoplasms and integrating geriatric testing in MDS and 3. a nursing forum in which all aspects of MDS from diagnosis, pathophysiology and treatment were reviewed including supportive care.

Dr. Malcovati provided a wonderful plenary lecture at the opening ceremony entitled 'MDS today and in 20 years' and gave us a glimpse of the personalized approaches to MDS that are upon us now and in the future.

The plenary sessions encompassed talks about the bone marrow microenvironment inflammation and immune pathogenesis of MDS, molecular mechanisms of disease and targets, low and high risk MDS, cellular and immune therapy, pediatric and hereditary MDS, difficult clinical cases, targeted agents, prognosis and predictive models, CMML and the spliceosome, and disease progression biology. We were provided the first glimpse of the long-awaited IPSS-Molecular (IPSS-M) by Dr. Elli Papaemmanuil and enjoyed debates conducted by Drs. Garcia-Manero and Malcovati about whether personalized therapy is ready for prime time in MDS and Drs. Fenaux and Heuser whether therapy related MDS is always a poor prognosis. We were educated about the critical importance

of germline predisposition to MDS and the mechanisms of clonal evolution from bone marrow failure by Drs. Shimamura, Godley and Makishima.

New to this meeting were 17 three-minute poster pitches provided by selected registrants highlighting the top ranked posters that were embedded into the plenary sessions together with 9 top ranked oral abstracts as well.

Early risers enjoyed 'meet the expert' educational talks provided by Dr. Lin about optimizing transfusion support for patients with MDS, Dr. Santini about lenalidomide treatment in MDS, Dr. Ebert about 'making heads and tails of NGS printouts and their role in MDS' and by Dr. Kulasakararaj who reviewed the overlap of AA, PNH and LGL.

The session finished off with the Tito Bastianello and MDS Foundation Investigator Awards who provided brief presentations of their award-winning abstracts. At any time, delegates could peruse the 56 e-posters on the online platform.

Finally, people who missed some of the talks or wanted to consolidate their knowledge from this whirlwind meeting had the opportunity to get abridged 'COLES-NOTES' summaries of the talks provided in the 'greatest hits from this meeting' final session provided by Drs. Buckstein, Yee and Schuh.

The meeting also offered Industry pipeline sessions in which the oral HMA pipeline was discussed (Astex), Tamibarotene, the RARA



#### **GUEST EDITORIAL**

agonist's efficacy in selected MDS patients (Syros) and the Gilead-Kite pipeline of compounds in hematology and oncology with a focus on magrolimab (Gilead). Other hosted industry sessions included lower risk MDS management including ESA's and luspatercept (Bristol Myers Squibb), immune based treatments for higher risk MDS (Novartis) and current and emerging treatments for MDS in 2021 (MediCom).

We are very grateful with the financial support provided by AbbVie, Acceleron Pharma, Astellas Oncology, Astex Pharmaceuticals, Bristol Myers Squibb, Gamida Cell, Gilead Sciences, MediCom Worldwide, Novartis, Silence Therapeutics, Syros Pharmaceuticals, Taiho Oncology and Takeda Pharmaceuticals as well as our partners RUNX-1 Research Program, MDS Foundation, MDS-Alliance and MDS-Europe organizations.

The co-chairs are indebted to Mr. Perry Gil-Ran and the Kenes team who helped plan the meeting, designed the very attractive and user-friendly online platform, operated the online sessions seamlessly and kept everything on track, and to the MDS Foundation (Mrs. Tracey Iraca and Ms. Lea Harrison) who helped organize the entire event over the last years. We also want to thank the scientific advisory committee who helped plan the program and vet the submitted abstracts.

While we were disappointed not to meet face to face in Toronto, we are optimistic that the 17th meeting scheduled for Marseille 2023 will finally allow us to socialize, discuss, debate and plan in person.

WE ARE OPTIMISTIC THAT THE

17TH MEETING SCHEDULED

FOR MARSEILLES 2023 WILL

FINALLY ALLOW US TO

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AND PLAN IN PERSON.





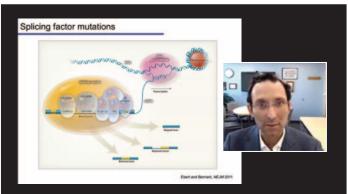


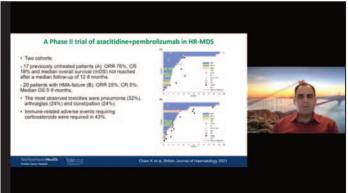












## HIGHLIGHTS FROM THE 16TH INTERNATIONAL SYMPOSIUM ON MYELODYSPLASTIC SYNDROMES

September 23–26, 2021 Toronto/Virtual









#### YOUNG INVESTIGATOR GRANT AWARDS

#### CONGRATULATIONS TO OUR 2021 YOUNG INVESTIGATOR GRANT WINNERS



ADITI SHASTRI, MD

Gilbert Bruce Smith Young Investigator Grant Winner

Funded by: MDS Foundation, Inc.

Grant Year: 2021-2023

Research Center: Albert Einstein College of Medicine Research Title: STAT3 Degradation to Overcome Therapy Resistance in MDS

Summary: MDS arises from the accumulation of mutations in hematopoietic stem cells (HSC's) & therapy resistance is invariable. We identified significant upregulation, increased expression of STAT3 in MDS-HSC's that was predictive of adverse prognosis. KTX-21 & KTX-105 are two specific STAT3 degraders that decreased cellular proliferation, and caused significant downregulation of STAT3 as well as its target genes (MCL1) in multiple hypomethylating agent and venetoclax resistant leukemic lines. In Aim 1, we test the efficacy of the STAT3 degraders by treating a large cohort of therapy resistant primary patient samples and PDX's. In Aim 2 we will evaluate the preclinical efficacy of STAT3 degradation alone and in combination with the clinically relevant MCL1 inhibitor AZD5991 in therapy resistant MDS.



#### SYED MIAN, PhD

Gilbert Bruce Smith Young Investigator Grant Winner

**Funded by:**MDS Foundation, Inc.

Grant Year: 2021-2023

Research Center: The Francis Crick Institute, Haematopoietic

Stem Cell Laboratory

Research Title: Identification and functional screening to identify niche-related therapeutic targets in Myelodysplasia

Summary: Myelodysplastic syndromes are a collection of clonal haematopoietic stem cell (HSCs) disorders with very limited treatment options. We hypothesise that a combination of aging and genetic abnormalities in HSCs transmit disease cues to the bone marrow niche that in-turn provides nurturing signals for the sustenance of the disease. A combination of xenotransplantation, RNA sequencing and cytokine profiling will be used to delineate the interacting surface proteins between the MDS HSCs and niche mesenchymal stromal cells. Large-scale siRNA screening followed by targeted inducible shRNA lentiviral approach will be used to identify the receptor-ligands that can be potentially used as therapeutic targets.

## **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. The 17th International Congress will be held in Marseille, France on May 3-6, 2023. We are also looking forward to our 3rd Regional Symposium on MDS 4-5 November 2022 in Kyoto, Japan. Our prior Regional Symposia were held in São Paulo, Brazil and Tel-Aviv, Israel.

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



#### **MEETING HIGHLIGHTS AND ANNOUNCEMENTS**

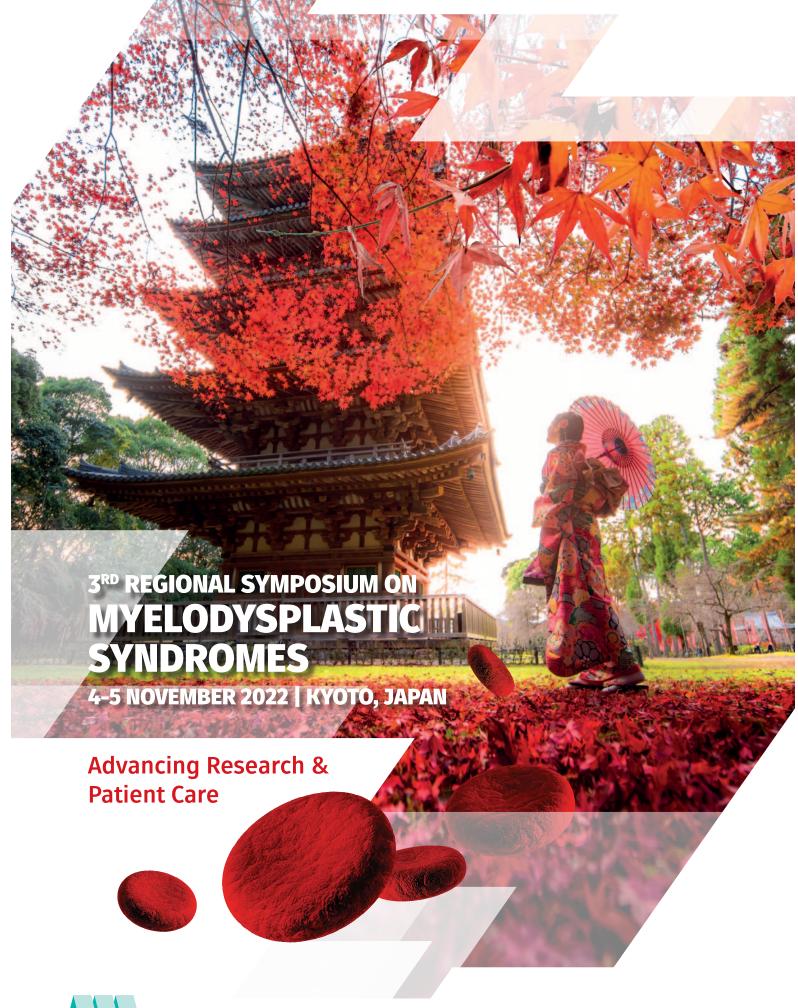


#### THANK YOU TO ALL WHO ATTENDED OUR ASH 2021 VIRTUAL SYMPOSIUM!

PRESENTATIONS CAN BE VIEWED ON-DEMAND AT:
HTTPS://WWW.MDS-FOUNDATION.ORG/ASH-2021-WEBINARS/









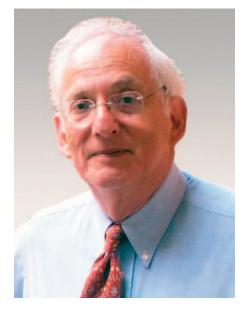
#### **INTERNATIONAL** WORKING GROUPS

#### MDS FOUNDATION INTERNATIONAL WORKING GROUP FOR PROGNOSIS IN MDS

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

The Molecular Committee of the International Working Group for Prognosis in MDS (IWG-PM) has been working to develop a clinical-molecular risk model (IPSS-Molecular or IPSS-M). To this end mutations in diagnostic MDS samples from 2957 patients from 13 countries and 25 global centers were analyzed. These data were presented by Elli Papaemmanuil and Elsa Bernard at the Toronto MDS Symposium, the IWG-PM group meeting, and at the 2021 American Society of Hematology meeting.<sup>1</sup> Clinical, cytogenetic, and molecular variables were evaluated for associations with leukemic transformation and overall survival. At least one genetic driver alteration found in 94% of patients. Multivariate analysis identified multihit TP53, FLT3 mutation, and MLL partial tandem duplication as top genetic predictors of adverse outcomes. SF3B1 mutation was associated with favorable outcomes, but this was modulated by co-mutation patterns. Using hematologic, cytogenetic and molecular data on 31 genes, the IPSS-M was developed as a continuous score. A discrete six-category risk schema was further derived. The IPSS-M re-stratified 46% of MDS patients compared to the IPSS-R, improving discrimination across clinical endpoints. A web calculator was built that, upon entering predictor variables, outputs a patient-tailored score, its corresponding risk category, and temporal estimates for clinical endpoints. The IPSS-M prognostic risk score is personalized, interpretable and reproducible. Combining conventional parameters with genomic profiling, the IPSS-M represents a valuable tool for clinical decision-making for MDS patients.

Other ongoing aims for the Molecular Project, discussed at the IWG-PM 2021 ASH meeting, included generating data for an MDS Classification model, genetic predictors





of response to HMAs, and analysis of mechanisms of disease progression obtained from sequential profiling.

At the Toronto IWG-PM group meeting, updates were also provided by Andrea Pellagatti regarding his project, Gene expression in MDS HSPCs using single cell analysis: Disease pathophysiology and outcome prediction, and by Andrea Kuendgen regarding cytogenetic features from the treatment-related MDS project.

Also reviewed were publications related to recent efforts by members of the group:

- Bernard E, Tuechler H, Greenberg PL, et al. EbertB, Bejar R, Malcovati L, Cazzola M, Ogawa S, Hellström-Lindberg E, Papaemmanuil E. A Clinical-Molecular and Personalized Risk Scoring System for Patients with MDS (IPSS-M), Proceedings ASH 2021, Atlanta, December, #61.
- Haase DT, Stevenson K, Neuberg D, Maciejewski J, et al, Bejar R. TP53 Mutation Status Divides MDS with Complex Karyotypes into Distinct Prognostic Risk Groups. Leukemia. 2019; 33:1747–1758.

- Bernard E, Nannya Y, Hasserjian, et al, Papaemmanuil E. Implications of TP53 Allelic State for Genome Stability, Clinical Presentation and Outcomes in MDS. Nature Medicine. 2020;26:2549–2556.
- Papemmanuil E, Classification and personalized prognosis in MDS. MDS Foundation Symposium, ASH meeting, 2019 Orlando, December.
- Malcovati L, Stevenson K, Papaemmanuil E, Neuberg D, Bejar R, et al, Cazzola M. SF3B1-mutant MDS as a distinct disease subtype — A Proposal IWG-PM. Blood. 2020;136:157–1704.
- Kuendgen A, Tuechler T, Nomdedeu M, et al, Sanz G. Therapy-related MDS deserve specific diagnostic subclassification and risk-stratification — An approach to classification of t-MDS. Leukemia. 2021:35,835–849.
- Nomdedeu M, Tuechler H, Kuendgen A, et al, Haase D. Cytogenetic findings in therapy-related MDS—relation with primary disease and therapy. Proc MDS Foundation Symposium, Copenhagen, May 2019, #127.



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

#### THE MDS/MPN INTERNATIONAL WORKING GROUP



MICHAEL SAVONA
Vanderbilt University Medical Center
Nashville, Tennessee

The MDS/MPN International Working Group (MDS/MPN IWG) was developed in 2012. By the end of 2013, membership was expanded. Work began to develop specialized disease response criteria for MDS/MPN and to begin a multi center project investigating the biology of CMML. The overarching goal of the MDS/MPN IWG is to identify key knowledge gaps in the area of MDS/MPNs and facilitate international, collaborative, translational science geared to rapidly improve our understanding of these complex, uncommon myeloid neoplasms. The current membership includes over 100 investigators, from across 12 countries.

In 2008 the World Health Organization designated distinct clinical entities with overlapping dysplastic and proliferative features. These include Chronic Myelomonocytic Leukemia (CMML), atypical CML (aCML), Juvenile Myelomonocytic Leukemia (JMML), and Myelodysplastic/Myeloproliferative Neoplasms Unclassifiable (MDS/MPN-U). The provisional entity of MDS/MPN with ring sideroblasts and thrombocytosis (MDS/MPN RST) has since joined this group, and many investigations have confirmed unique molecular under-pinnings and clinical

trajectories of each of these diseases. Nonetheless, this stratification of rare myeloid neoplasms requires collaborative efforts to make transformative changes in patient care.

Since the publication of the Proposed MDS/MPN Response Criteria in 2015, 1 the clinical trial opportunities for patients with MDS/MPN have dramatically increased. This led to development of MDS/MPNspecific trials led by MDS/MPN IWG members,<sup>2</sup> specifically, the first MDS/MPN IWG International study, ABNLMARRO, an international basket study designed to allow new compounds and combinations of therapy to be introduced easily among MDS/MPN IWG clinical sites which have expertise managing MDS/MPN patients, study the biology and pathophysiology of the diseases, and have multilateral expertise in this area. ABNL MARRO-001 is the first MDS/MPN IWG study and is currently underway. In addition to semi-annual meetings at ASH and EHA, the MDS/MPN IWG often conducts biennial meetings to focus international efforts in MDS/MPN.

Additional efforts in the MDS/MPN IWG center around growing new models of disease. Recently, robust patient-derived xenografts transplant models for CMML were shown to be the first published reliable means to study specific genetic lesions in MDS/MPN in animals.<sup>3</sup> Advances have also been made in molecular testing and diagnostics which allow tracking of specific mutations at diagnosis and with treatment.<sup>4</sup> The means by which these mutational changes are able to define prognosis and effect treatment are the focus of the work of many MDS/MPN IWG members.

ADDITIONAL EFFORTS IN

THE MDS/MPN IWG

CENTER AROUND GROWING

NEW MODELS OF DISEASE.



#### **Peer-Reviewed Publications**

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

#### **Ongoing Collaborations**

- Expansion and prospective molecular sequencing of the international CMML data set.
- Exploring the consequence of an MDS/MPN diagnosis on quality of life.
- Identify/Generating a consensus CMML prognostic model.
- Exploring the role of transplant in molecularly defined CMML subtypes.
- Implementing international clinical trials on both sides of the Atlantic.

#### References:

- Savona, M. R. et al. An international consortium proposal of uniform response criteria for myelodysplastic/myeloproliferative neoplasms (MDS/MPN) in adults. Blood. 125, 1857-1865, doi:10.1182/blood-2014-10-607341 (2015).
- Mughal, T. I. et al. An International MDS/MPN
  Working Group's perspective and recommendations on molecular pathogenesis, diagnosis and clinical characterization of myelodysplastic/myeloproliferative neoplasms. Haematologica. 100;1117-1130, doi:10.3324/haematol. 2014.114660 (2015).
- Padron, E. et al. An international data set for CMML validates prognostic scoring systems and demonstrates a need for novel prognostication strategies. Blood cancer journal 5, e333, doi:10.1038/bcj.2015.53 (2015).

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#### 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.



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





#### Why share your experiences?

To allow patients, carers and loved ones to learn more about their condition and to help others.



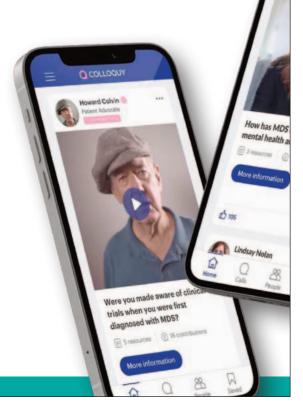
#### How can you help others?

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



#### Guided by you!

Colloquy is guided by an ambassador group of patients, carers, and experts, who ensure the patient perspective is brought to the forefront of the community.



## **QUESTIONS ANSWERED**



## VISIT OUR NEW MICROSITE



#### 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.

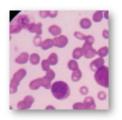
#### **MDS SUBTYPE**

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.



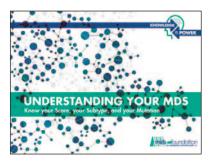
BONE MARROW BLAST



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/.



## JOIN THE MOVEMENT in 2022

Join your local Move for MDS community walk, fundraise and show support to the Myelodysplastic Syndromes Foundation in our fight against MDS. 100% of every \$1 donated goes towards increasing awareness and accelerating critical research.

#### **Dates & Locations**

#### LOS ANGELES

**Date:** August 28th, 2022 **Target Location:** Griffith Park

#### CHICAGO

**Date:** September 25th, 2022 **Target Location:** Soldier Field or

Maggie Daley Park

#### **NEW YORK CITY**

Date: October 2nd, 2022

Target Location: Battery Park City Promenade

#### NASHVILLE

**Date:** October 15th, 2022 **Target Location:** TBD

#### BOSTON/GLOBAL

Date: October 23rd, 2022

Target Location: Boston Common



For more information visit MoveForMDS.org

## **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













Presentation By:



Courtney D. DiNardo, M.D., MSCE The University of Texas MD Anderson Cancer Center

#### **Medical Education Webinar**

#### Hereditary Hematologic Malignancies: Not That Rare

#### **Learning Objectives**

- · Understanding the role of genetic testing on patient outcomes for HM patients.
- Learning the prevalence of pathogenic germline variants in HM patients.
- · Gaining tools on how to suspect and diagnose hereditary HM.
- · Learning how to manage hereditary HM patients and their at-risk family.

#### This webinar is offered on two dates with different discussion panelists:

Monday, May 23rd, 2022 1:00pm PDT | 3:00pm CDT | 4:00pm EDT



Amy DeZern, M.D., M.H.S. Sidney Kimmel Comprehensive Cancer Center Johns Hopkins School of Medicine



Vinod A Pullarkat, M.D.

City of Hope

Comprehensive Cancer Center



Michael R. Savona, M.D. Vanderbilt-Ingram Cancer Center Vanderbilt University School of Medicine

Yasmin Abaza, M.D.
Robert H. Lurle
Comprehensive Cancer Center
Northwestern University



Wednesday, May 25th, 2022

8:00am PDT | 10:00am CDT | 11:00am EDT

Kelly Bolton, M.D., Ph.D. Alvin J. Siteman Cancer Cente Washington University



Elizabeth Griffiths, M.D. Roswell Park Comprehensive Cancer Center SUNY at Buffalo

Register for May 23 here: https://bit.ly/mMay2322HHM

Register for May 25 here: https://bit.ly/mMay2522HHM

#### LITERATURE HIGHLIGHTS

## HIGHLIGHTS OF LATEST LITERATURE IN MDS

#### SUNEEL D. MUNDLE, PHD RAMA BHAGWAT

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

## EPIDEMIOLOGY, DIAGNOSIS AND PROGNOSIS:

 Pulte D, et al. Incidence and survival estimates for patients with myelodysplastic syndrome in the 21st century: no evidence of improvement over time. Leuk Lymphoma. 2022; Mar 27 [Online ahead of print] (DOI:10.1080/10428194.2022. 2053529)

The incidence and survival analysis of data from the SEER-18 database in the USA for a period of 2001-2016 demonstrated a peak incidence of MDS in 2010 at 5.6 per 100,000, which decreased subsequently to 3.9 by 2016. When survival trends were examined between 2008-2016, a trend of reduced 1 yr-, 2 yr- and 5 yr- survival was noted in 2014-2016 period particularly in patients >55 years of age. While in those <55 yr, the survival rates remained unchanged over the period of 2008-2016. The authors highlighted a continued unmet need for newer therapies to improve prognosis of MDS.

 Yanagisawa, et al. Prognostic impacts of serum levels of C-reactive protein, albumin and total cholesterol in patients with myelodysplastic syndromes. Int J Hematol. 2022; Mar 23 [Online ahead of print] (DOI: 10.1007/s12185-022-03321-z)

An assessment of the prognostic impact of laboratory findings in 123 patients with MDS or MDS/MPN at a single institution between 2010 and 2020, showed that overall survival was significantly shorter in patients with High C-reactive protein or low albumin or low total cholesterol at baseline. In a multivariable analysis,



serum C-reactive protein retained the prognostic significance.

- Radakovich N, et al. A geno-clinical decision model for the diagnosis of myelodysplastic syndromes. *Blood Adv.* 2021;5(21):4361-4369. (DOI: 10.1182 /bloodadvances.2021004755)
  - The model described in this report combines next generation sequencing data with clinically important phenotype information and using machine learning the proposed model develops clinicogenomic relationship algorithms. This is expected to provide a significant improvement in differential diagnosis of myeloid malignancies independent of bone marrow biopsy data.
- Kaivers J, et al. The WHO 2016 diagnostic criteria for acute myeloid leukemia with myelodysplasia related changes (AML-MRC) produce a very heterogeneous entity: A retrospective analysis of the FAB subtype RAEB-T. Leuk Res. 2022;112:106757. (DOI: 10.1016/ j.leukres.2021.106757)

The study included cytomorphologic review of 79 patients with AML with myelodysplasia related changes or RAEB-T. Only 30% fulfilled morphologic WHO criteria, while 46.8% were classified based on antecedent MDS and 54.4% were diagnosed due to detection of MDS related cytogenetic abnormalities. Particularly, those with MDS related cytogenetic abnormalities, demonstrated poorer survival compared to others (approx. 8 mo vs 14 mo, p=0.026). Also, the criterion of ≥50%

dysplasia in ≥2 lineages was found to be less helpful, which is therefore recommended to be deleted in future update of WHO guidelines.

#### **TREATMENT:**

#### **RBC Transfusion and Growth Factors:**

- 1. Vetro C, et al. Response assessment to erythropoietin-zeta (Epo-Alpha biosimilar) therapy in low-risk myelodysplastic syndromes. J Clin Med. 2022;11(6): 1665. (DOI: 10.3390/jcm11061665) The study enrolled 36 patients with low/Int-1 MDS patients having <500U/L serum erythropoietin (EPO). Erythropoietin-zeta was administered at 40,000U once a week. Eighteen patients (50%) showed significant improvement in Hb (≥1.5g/dL) in 8 weeks and an additional 4 patients (11%) showed similar response in 16 weeks. While 9 patients needed double the dose (40,000 U twice a week) to show erythroid response. 24/36 patients were followed up for a median of 5.3 yr with a 5 yr survival rate of 89%. The safety was found to be consistent with prior experience with erythropoiesis stimulating agents.
- Farrukh F, et al. Real-world experience with luspatercept and predictors of response in myelodysplastic syndromes with ring sideroblasts. Am J Hematol. 2022; Aug 16 [Online ahead of print] (DOI: 10.1002/ajh.26533)

This retrospective single institution study assessed clinical benefit of Luspatercept in real life patients (n=39) primarily with low/very low risk (87%), transfusion

#### LITERATURE HIGHLIGHTS

dependent MDS (80%), 95% of patients previously had ESAs and 38% and 26% hypomethylating agents lenalidomide respectively. At baseline, abnormal karyotype was noted in 39% and SF3B1, DNMT3A and TET2 mutations in 83%, 31% and 31% patients respectively. With a median follow up of lyr, median duration of luspatercept treatment was 4 mo (in responders 8 mo). Erythroid response was noted in 7/39 (18%) including 5/31(16%) transfusion dependent patients achieving transfusion independence (med duration of response 6 mo). Multivariate analysis demonstrated prognostic impact of elevated serum EPO levels (>80IU/L).

#### **Hypomethylating Agents:**

- 1. Hernandez-Boluda JC, et al. Acute leukemia arising from myeloproliferative or myelodysplastic/myeloproliferative neoplasms: A series of 372 patients from the PETHEMA AML registry. Leuk Res. 2022; Mar 6 [Online ahead of print] (DOI: 10.1016/j.leukres.2022.106821) This retrospective study evaluated disease characteristics, treatments and outcomes over 27 years in 372 AML patients that evolved after myeloproliferative neoplasms (MPN) or MDS/MPN. 38% of patients received intensive chemotherapy, 17% got HMA and 14% had non-intensive chemotherapy. Although CR was higher with intensive chemotherapy (43%), the response was short lived without subsequent hematopoietic cell transplant. Those treated with intensive chemotherapy (8.5 mo) or HMAs (8.6 mo) had longer survival compared to those with non-intensive chemotherapy (4.2 mo). Also, transplantation in CR vs in other response categories showed a better 3yr survival rate (64% vs. 22% respectively, p=0.002).
- Graveno ME, et al. Venetoclax in combination with hypomethylating agents or low dose cytarabine for relapsed and refractory acute myeloid leukemia. Leuk Lymphoma. 2022; Mar 8 [Online ahead of print] (DOI:10.1080/10428194. 2022.2042688)

A retrospective analysis of 77 patients with R/R AML or AML secondary to MDS with median 1 prior therapy who were treated with venetoclax plus HMA or low dose cytarabine combination. The median -OS was 13.1 mo, -PFS 12 mo, and duration of response was 8.9 mo. Overall response rate was 68% with CR/CR with incomplete hematopoietic recovery was 53%.

#### **Targeted Therapies:**

- Zeidan AM, et al. A phase 1/2 study of the oral Janus Kinase 1 inhibitors INCB052793 and Itacitinib alone or in combination with standard therapies for advanced hematologic malignancies. Clin Lymphoma Myeloma Leuk. 2022; Jan 20 [Online ahead of print] (DOI: 10.1016/j.clml.2022.01.012)
  - A phase 1/2 study evaluated two novel JAK1 inhibitors. Overall, fifty-eight patients participated across phase 1a, 1b and 2 of the study. Clinical response was rare, and all patients discontinued either due to progression or adverse event. In phase 2 AML and MDS patients that had prior HMA exposure were treated with JAK1 inhibitors in combination with Azacytidine (INCB052793+AZA, n=9; Itacitinib+AZA, n=10). During phase 2 assessment, 1/3 MDS patients showed a marrow CR and no AML patient showed response to INCB052793+AZA. While 1 patient with AML and none with MDS showed objective response Itacitinib+AZA. The toxicity profile was primarily linked to myelosuppression. The study was terminated due to limited clinical benefit.
- Gerds AT, et al. Atezolizumab alone or in combination did not demonstrate a favorable risk-benefit profile in myelodysplastic syndrome. Blood Adv. 2022;6(4):1152-1161. (DOI: 10.1182/ bloodadvances.2021005240)
  - A PD-L1 inhibitor, Atezolizumab was studied as a monotherapy or in combination with AZA in patients with R/R or HMA-Naïve MDS (n=46). Three cohorts were studied- (A) Atezolizumab monotherapy in R/R MDS, (B) in

- combination with azacytidine in R/R MDS or (C) HMA-naïve patients. In three cohorts, the rate of death was 64%, 79% and 62% respectively. While no patient showed clinical response in cohort A, 2 patients responded in cohort B and 13 responded in cohort C. The B/R however was unfavorable across all three cohorts.
- 3. DiNardo CD, et al. Enasidenib plus azacitidine versus azacitidine alone in patients with newly diagnosed, mutant-IDH2 acute myeloid leukemia (AG221-AML-005): a single-arm, phase 1b and randomized phase 2 trial. Lancet Onc. 2021;22(11):1597-1608. (DOI: 10.1016/\$1470-2045(21)00494-0) In Phase 1b, two doses of enasidenib 100 or 200 mg/day were assessed in a 28day cycle with azacitidine. Subsequently, in phase 2 study the combination was randomized (2:1) again AZA alone with primary end point of overall response rate. 107 of the total 322 AML patients screened had IDH2 mutations. No dose limiting toxicities were identified in 6 patients enrolled in phase 1b and 100 mg/ day was the recommended dose for phase 2. The overall response rate was noted in 50/68 (74%) with enasidenib+ AZA versus 12/33(36%) with AZA alone (Odds Ratio 4.9, p=0.0003). The serious grade 3/4 adverse events with enasidenib+AZA versus AZA alone were thrombocytopenia (37% vs 25%), anemia (19% vs 22%) and febrile neutropenia (16% vs 16%).

#### **Novel Therapies:**

- Steensma DP, et al. Phase I first-in-human dose escalation study of the oral SF3B1 modulator H3B-8800 in myeloid neoplasms. Leukemia. 2021;35(12): 3542-3550. (DOI:10.1038/s41375-021-01328-9)
  - In a phase I study of SF3B1 binding small molecule, two dosing schedules were assessed: 5 days on/9 days off and 21 days on/7 days off. Of the total 84 MDS, CMML or AML patients (75% RBC transfusion dependent), 27 had received treatment ≥6 mo. Treatment emergent adverse events included diarrhea,

#### LITERATURE HIGHLIGHTS

nausea, fatigue, and vomiting. Five of the 15 MDS patients with missense SF3B1 mutations experienced RBC transfusion independence.

1. Gbyli R, et al. In vivo anti-tumor effect of

#### Pathobiology:

- PARP inhibition in IDH1/2 mutant MDS/AML resistant to targeted inhibitors of mutant IDH1/2. Leukemia. 2022; Mar 10 [Online ahead of print] (DOI: 10.1038/s41375-022-01536-x)

  Primary xenografts (PDX) derived from patients with IDH1/2-mutant AML/MDS were established in which poly-ADP ribose polymerase (PARP)- inhibitor, Olaparib, was shown to reduce the AML/MDS cell engraftment and leukemia initiation in IDH1/2 mutant, but not in wildtype cells. For IDH1/2 mutant cells, Olaparib
- 2. Sahoo SS, et al. Clinical evolution, genetic landscape and trajectories of clonal hematopoiesis in SAMD9/SAMD9L syndromes. Nature Med. 2021;27: 1806-1817. (DOI: 10.1038/s41591-021-01511-6)

or resistant PDXs.

inhibited both IDH-mutant inhibitor naïve

In a clinically annotated pediatric MDS cohort (n=669) among the consecutively diagnosed cases, germline SAMD9/9L mutations were found in 8% patients and were exclusive with GATA2 mutations in 7%. Among patients with SAMD9/9L, 90% had refractory cytopenia, 28% immune dysfunction, 38% acquired monosomy and 57% patients showed constitutional abnormalities.

## Reviews, Perspectives, Case Reports and Guidelines

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

- Ramos F. Myelodysplastic syndrome: Riding the crest of the wave. J Clin Med. 2022;11(6):1606.(DOI:10.3390/ jcm11061606)
- 2. Van de Loosdrecht, et al. EHA endorsement of ESMO clinical practice

- guidelines for diagnosis, treatment, and follow-up for myelodysplastic syndromes. HemaSphere. 2022;6(3):e695. (DOI: 10.1097/HS9.000000000000000695)
- Soper J. Patient and caregiver insights into the disease burden of myelodysplastic syndrome. Patient Relat Outcome Meas. 2022;13:31-38.(DOI:10.2147/ PROM. S346434)
- Greenberg PL, et al. NCCN guidelines® Insights: Myelodysplastic Syndromes, Version 3.2022. J Natl Compr Canc Netw. 2022;20(2):106-117. (DOI: 10.6004/jnccn.2022.0009)
- Greenberg PL. Metchnikoff's legacy: the dysplastic nature of innate immunity in myelodysplastic syndromes. Haematologica. 2022;107(3):568-569.(DOI: 10.3324/ haematol.2021.279419)
- Lipof JJ, et al. A case of acute eosinophilic leukemia with a novel PHF6 mutation. Case Rep Hematol. 2021:5574766. (DOI: 10.1155/2021/5574766)
- 7. Jain AG, Zhang L, Benett JM and Komrokji R. Myelodysplastic syndromes with bone marrow fibrosis: An update. *Ann Lab Med*. 2022;42(3):299-305. (DOI: 10.3343/alm.2022.42.3.299)

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

## Matterhorn DO YOU HAVE MDS WITH ANEMIA?

If you have been diagnosed with myelodysplastic syndrome (MDS) with anemia, the Matterhorn clinical trial is a research study investigating a potential treatment to help treat your anemia and potentially reduce your need for blood transfusions.



Learn more at ClinicalTrials.gov NCT03263091



Myelodysplastic Syndromes is a rare blood cancer.

The MDS
Foundation, Inc.
supports and
educates patients,
their communities,
and healthcare
providers.





#### 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

#### MDS RISK ASSESSMENT CALCULATOR

THE IWG-PM UNDER THE AEGIS OF THE MDS FOUNDATION, INC. DEVELOPED A PROGNOSTIC TOOL, THE IPSS-R CALCULATOR, TO DETERMINE A PATIENT'S RISK OF PROGRESSING TO ACUTE MYELOID LEUKEMIA (AML).

## **BASIC**CALCULATOR:

https://www.mds-foundation.org/ ipss-r-calculator/

## ADVANCED CALCULATOR:

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



#### **APPLE STORE:**

https://apps.apple.com/app/id585168085



#### **GOOGLE PLAY STORE:**

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



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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 123 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.
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- In addition, along with your \$500 CoE renewal payment, your annual MDSF Professional Membership dues are waived.
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- 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
- Recognized morphologic expertise in MDS
- Available cytogenetics and/or molecular genetics
- Ongoing research, including Institutional Review Boardapproved clinical trials
- Documentation of peer-reviewed publications in the field

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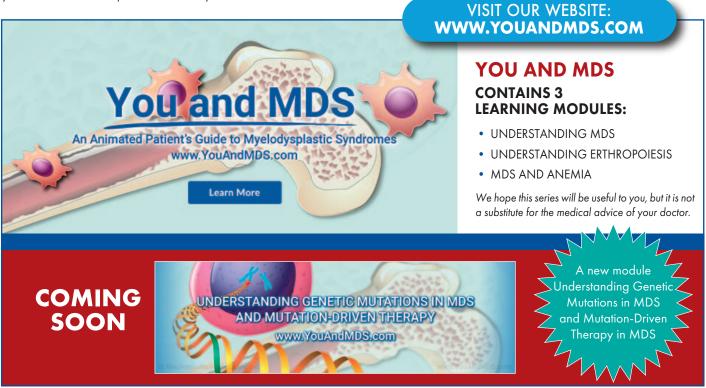
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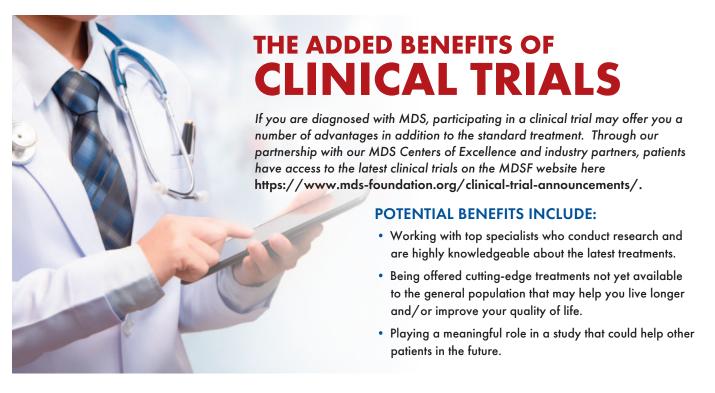
#### **PATIENT** RESOURCES

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

#### YOU AND MDS

This resource is intended for patients with MDS, as well as family members and caregivers. You will find expert advice about MDS to help you discuss key issues with your health care provider and make important decisions related to management and treatment. Easy-to-understand animations with audio narration, expert video explanations, patient interviews, illustrated slide shows, and educational downloads are available to you. Also available in Spanish www.ustedysmd.com.





# 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/registry25



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:

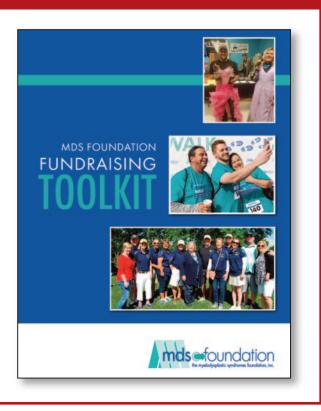
https://bit.ly/3Kx4B8e

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 2021, we held five **Move For MDS** walks that took place in the Pacific Northwest, California, Chicago, New York City, and Boston/Global and we're thrilled to share that we have

raised over \$565,000

to accelerate critical research of Myelodysplastic Syndromes.





#### 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.



We believe that the MDS Foundation is the heart of the MDS community, bringing together patients, caregivers, families, and physicians to take on this disease together. To be more inclusive of our community, we are discontinuing patient memberships\* because we're all one big MDS family, connected through diagnosis and beyond. We will continue to cater to all the members of our community and provide support and resources for education, advocacy, and treatment research.

Join our community to stay connected, receive updates, and learn about the ways you can get involved!

SIGN-UP FOR E-NEWS @MDS-FOUNDATION.ORG/



#### JOIN THE MDS FOUNDATION



#### 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.



#### JOIN THE MDS FOUNDATION

## THINKING OF JOINING THE MDS FOUNDATION AS A PROFESSIONAL MEMBER, COMMUNITY PROFESSIONAL MEMBER OR CHANGE THE FUTURE OF MDS PROFESSIONAL MEMBER?

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

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#### 2022 WEBINARS FOR MDS PATIENTS & CAREGIVERS

## WE ARE VIRTUAL!



We have planned a comprehensive series of webinars for 2022 bringing experts and the MDS community together to provide educational information, best practices, tools, and resources

Whether you are a newly diagnosed patient, a long-term survivor, or caregiver, our webinar series will have something for you.

We have collaborated with renowned hematology professionals who will address key topics and questions you may have using language that is easy to understand in a 90-minute format that will include live Q&A opportunities for all participants.



MISSED OUR LIVE WEBINAR?
VIEW PREVIOUS LIVE WEBINARS
AT A TIME THAT IS CONVENIENT FOR YOU!

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

"Aren't you scared?" a young person asked me. Sometimes, yes. I try to remember to pray when I am afraid. The MDS Foundation sponsors webinars that have helped me learn a lot. I remind myself that I am living with MDS, not dying with it."



## MDS FOUNDATION PODCASTS – NOW AVAILABLE!

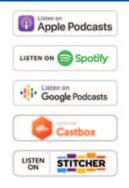


## THIS PODCAST SERIES PROVIDES IMPORTANT UP-TO-THE-MINUTE INFORMATION ON MDS INCLUDING DIAGNOSIS, TREATMENT AND CLINICAL RESEARCH.

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!

The MDS PROFESSIONAL REPORT will cover international meetings as well as recently published articles on MDS, combining educational materials with cutting-edge information. The format will include a description of studies by the editors, interviews with experts, conversations, round table discussions as well as other relevant formats. We are planning to include several episodes per year lasting 20–25 minutes covering a range of topics.



#### SEASON 2: EPISODE 1: MDS in ASH 2021 - Are We Already in the Molecular Era?

Our hosts, Moshe Mittelman, of The Tel Aviv Sourasky Medical Center, and Drorit Merkel, of the Sheba Medical Center, both from Tel Aviv University, Israel, discuss several important presentations from the recent 2021 ASH meeting.

#### Visit our website to listen to previous episodes:

**SEASON 1: EPISODE 1:** TP53 Mutations: Were They Born Equal?

SEASON 1: EPISODE 2: Don't Withhold Anti-Neoplastic Treatment from your Hematological Patients Infected by COVID

**SEASON 1: EPISODE 3: Will Venetoclax Become The New Standard?** 

SEASON 1: EPISODE 4: Do Immune Check Inhibitors have a Role in the Treatment of Higher-Risk MDS?

WE HOPE YOU FIND THE MDS PROFESSIONAL REPORT INFORMATIVE, INTERESTING, AND USEFUL.



## SYROS RECEIVES FDA ORPHAN DRUG DESIGNATION FOR TAMIBAROTENE FOR THE TREATMENT OF MDS

**CAMBRIDGE, MA – February 2, 2022 (BUSINESS WIRE).** Syros Pharmaceuticals (NASDAQ:SYRS), a leader in the development of medicines that control the expression of genes, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to 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).

"The FDA's orphan drug designation is an important milestone in the development of tamibarotene as a treatment for MDS," said David A. Roth, M.D., Syros' Chief Medical Officer. "We believe tamibarotene's novel mechanism of action, promising clinical activity data, oral delivery, and favorable tolerability profile supports a potential new option for the approximately 30% of HR-MDS patients who are RARA-positive. We are focused on developing the first potential therapy for a targeted population in HR-MDS as we continue to advance our ongoing SELECT-MDS-1 pivotal trial."

The FDA's Office of Orphan Drug Products grants orphan status to support development of medicines for the treatment of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug designation may provide certain benefits, including a seven-year period of market exclusivity if the drug is approved, tax credits for qualified clinical trials and an exemption from FDA application fees.

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. Safety lead-in data from the ongoing SELECT-AML-1 Phase 2 trial is expected in the second half of this year.

# GENOMIC TESTING COOPERATIVE AND THEIR COOPERATIVE MEMBER LABORATORIES EXPAND HEMATOLOGY PANELS TO COVER VEXAS DISEASE AND A NEW PROGNOSTIC BIOMARKER IN MYELOPROLIFERATIVE NEOPLASMS

**IRVINE, CA – March 2, 2022.** Genomic Testing Cooperative, LCA (GTC) announced today that their hematology molecular profiling is expanded to cover analysis of the UBA1 and NFE2 genes. Both genes were recently reported to be clinically important for the diagnosis and treatment of patients with hematologic neoplasms.

Recently described VEXAS (vacuoles, E1 enzyme, X-linked, autoinflammatory, somatic) is caused by mutations in the UBA1 gene. This is an adults-onset fatal disease that may present as myelodysplastic syndrome, aplastic anemia or multiple myeloma, but characterized by fevers, low white cell count, vacuoles in bone marrow cells, dysplastic bone marrow, pulmonary inflammation, chondritis, and vasculitis. Detecting the presence of mutations in the UAB1 gene is the only way for confirming the diagnosis of this disease.

The NFE2 gene is mutated in approximately 8–9% of patients with typical myeloproliferative neoplasms (MPN) and in 3% of triple negative myeloproliferative neoplasms as well as in isolated granulocytic sarcoma, some patients with myelodysplastic syndrome, and acute myeloid leukemia. Most importantly, the presence of mutations in the NFE2 gene in MPN is associated with transformation into acute myeloid leukemia and poor survival.

Testing for these two genes will be included when all hematology profiling tests are ordered from GTC laboratories as well as from the Co-Op member laboratories. These genes are now included in the three hematology tests: Hematology Profile (DNA only), Hematology Profile Plus (DNA+RNA) and Liquid Biopsy Hematology Profile.

Maher Albitar, MD, Founder, Chief Executive Officer and Chief Medical Officer, GTC, stated, "Genomic medicine is advancing at rapid pace and keeping up with these advances requires diagnostics to be agile and responsive." Dr. Albitar added "This is an example of how the cooperative business model works. Clinical needs were communicated by members of the Co-Op. GTC developed the technology around implementing the changes to the current panels. This is followed by the exchange of information and samples for rigorous crossvalidation and implementation of changes to panels in member laboratories in a seamless and costeffective fashion".

The prevalence of VEXAS in the general population is unknown, but it is expected to be high in individuals with chronic inflammatory diseases. Most of the patients currently diagnosed with VEXAS have had numerous tests and tried multiple treatments. VEXAS should be considered in patients with systemic autoinflammatory disorders as well as patients with clinical presentation of myelodysplastic syndrome and chronic myelomonocytic leukemia. The annual incidence rate of MPN is 2.34 per 100,000.

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CURIS ANNOUNCES
UPDATED DATA WITH
ADDITIONAL ENCOURAGING
CLINICAL ACTIVITY IN PHASE
1/2 STUDY OF CA-4948
MONOTHERAPY IN TARGETED
PATIENTS WITH RELAPSED OR
REFRACTORY AML AND MDS;
AND INITIAL CLINICAL DATA
FROM PHASE 1 STUDY OF CI8993 IN PATIENTS WITH
RELAPSED OR REFRACTORY
SOLID TUMORS

- 40% CR/CRh rate (complete remission and complete remission with partial hematologic recovery) in R/R AML patients with U2AF1 or SF3B1 spliceosome mutation treated with CA-4948
- 57% ORR (objective response rate) observed in R/R MDS patients with U2AF1 or SF3B1 spliceosome mutation treated with CA-4948
- Added potential benefit of FLT3 inhibition highlighted by significant marrow blast reduction and eradication

- of FLT3 mutation in 2 out of 3 R/R AML patients with FLT3 mutation at baseline following treatment with CA-4948
- Promising initial safety data of CI-8993 highlights effectiveness of procedures implemented to manage expected cytokine release syndrome and enable dose escalation past 0.3 mg/kg
- Pharmacodynamic data provide early indication that targeting VISTA with CI-8993 may activate multiple anti-cancer mechanisms

LEXINGTON, MA — January 6, 2022 (PRNewswire). Curis, Inc. (NASDAQ: CRIS), a biotechnology company focused on the development of innovative therapeutics for the treatment of cancer, today announced positive updated clinical data from the ongoing open label Phase 1/2 dose escalation and expansion study of CA-4948, a novel, small molecule IRAK-4 inhibitor, as a monotherapy in patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) or high risk myelodysplastic syndromes (MDS) as well as initial pharmacokinetic and pharmacodynamic data from the Phase 1 dose escalation study of CI-8993, a monoclonal antibody targeting VISTA for patients with R/R solid tumors.

"These data continue to build on what we believe to be a compelling profile for CA-4948, showing its activity as a monotherapy in a targeted population of patients living with R/R AML/MDS, for whom prior lines of therapy have been unsuccessful," said James Dentzer, President and Chief Executive Officer of Curis. "We are especially pleased that these results demonstrate both a favorable safety profile and improved anti-cancer activity compared with standard of care therapies for these patients. Furthermore, we have been able to successfully identify and enroll these patients using existing genetic diagnostic panels. We remain on track to enroll additional patients with a spliceosome mutation to prepare for potential discussions with the U.S. Food and Drug Administration (FDA) in the first half of 2022 regarding the potential for a rapid registrational path forward for CA-4948 as a monotherapy in genetically-defined patient populations."

"We are also encouraged by the safety data from the CI-8993 trial, which we believe demonstrate that the procedures we implemented to manage the expected cytokine release effect have been successful—and have allowed us to escalate patient dosing up to and beyond 0.3 mg/kg," he continued. "We have recently begun dosing at 0.6 mg/kg; and look forward to providing another update on our progress in the second half of 2022. We are thrilled to have achieved this key safety and dose escalation milestone, as it brings us one step closer to providing anti-VISTA therapy for patients living with solid tumors."

## Phase 1/2 monotherapy study of CA-4948 in R/R AML/MDS

#### Well Tolerated and Manageable Safety Profile at 300 mg BID Dose Level

As of December 16, 2021, 49 total patients had been administered CA-4948 in the R/R AML/MDS study across 200 mg, 300 mg, 400 mg, and 500 mg dose cohorts. The safety profile observed to date showed the following key findings:

- CA-4948 was well-tolerated across multiple dose levels, including at the Recommended Phase 2 Dose of 300 mg BID
- Treatment-related adverse events were reversible and manageable
- No dose-limiting myelosuppression
- No cumulative toxicities observed
- No grade 4 or 5 treatment-related adverse events

We believe these attributes of CA-4948's emerging safety profile may provide an advantage compared to current standard of care therapies in monotherapy and could also make CA-4948 an attractive candidate for addition to combination therapy regimens.

## In Expanded Data Set, Findings Support Earlier Data Presented in June 2021

Previous data presented by Curis at the European Hematology Association in June 2021, highlighted preliminary efficacy data of CA-4948 in R/R AML/MDS patients whose disease is characterized by

spliceosome or FLT3 mutation. It is this genetically-defined subset of AML/MDS that is specifically targeted by CA-4948 and therefore represents the patients most likely to benefit from treatment with CA-4948 in monotherapy. Today's clinical data update provides an expanded data set for this genetically-defined patient population and further support the rationale for seeking a discussion with FDA in the first half of 2022 to discuss the potential for a rapid registrational path forward for CA-4948 as a monotherapy in genetically-defined patient populations.

In order to assess preliminary efficacy for these patients on study, Curis presented data on patients that had enrolled as of September 30, 2021, which allowed the opportunity for at least 2 disease assessments, to determine marrow response. Based on this criterion, there were 12 evaluable patients with a U2AF1 or SF3B1 spliceosome mutation (7 MDS; 5 AML) and 3 evaluable patients with a FLT3 mutation. There were 13 total evaluable patients; two AML patients presented with both a spliceosome mutation and FLT3 mutation and are therefore included in both subpopulations. These patients experienced a median of 2 prior lines of therapy (range 1-4), and all patients had prior hypomethylating agent (HMA) treatment.

In patients with spliceosome-mutated R/R AML, key findings included:

- CR/CRh rate of 40% (2 out of 5 patients)
- Both patients who achieved CR/CRh have been on study >6 months and achieved negative MRD (minimal residual disease) status
- Consistent tumor burden reduction observed, 3 out of 5 patients with elevated blast counts achieving ≥ 50% reduction in blast counts

In patients with spliceosome-mutated R/R MDS, key findings included:

 Objective response rate of 57% (4 out of 7 patients)

- One of the patients who achieved a marrow CR (mCR) proceeded to stem cell transplant after 1 cycle
- Consistent tumor burden reduction observed, with 4 out of 7 patients achieving ≥ 50% reduction in blast counts

In patients with a FLT3 mutated R/R AML, key findings included:

- CR rate of 33% (1 out of 3 patients)
- 2 out of 3 patients showed eradication of FLT3 mutation following treatment, indicating potential to modify the disease
- Consistent tumor burden reduction observed; with 2 out of 3 patients with elevated blast counts achieving ≥50% reduction in blast counts

We believe the data suggest a favorable safety and anti-cancer activity profile compared to standard of care therapies for these patient populations.

Enrollment in the study of CA-4948 in R/R AML/MDS is on-going, and Curis looks forward to potential discussions with the FDA in the first half of 2022 regarding the potential for a rapid registrational path forward for CA-4948 as a monotherapy in genetically-defined patient populations. Curis expects to provide additional data from the R/R AML/MDS study at a medical meeting in 2022.

## Phase 1 monotherapy study of CI-8993 in R/R solid tumors

#### Promising Safety Profile - No DLTs

Based on 13 patients treated in the first two dose cohorts of 0.15 mg/kg and 0.3 mg/kg, we believe CI-8993 has shown a promising safety profile to date, with no dose-limiting toxicities observed.

Following the implementation of safety measures including step dosing and co-medication, the trial has successfully dose escalated through the 0.15 mg/kg and 0.3 mg/kg cohorts, the dose level at which Janssen discontinued a prior study after a patient experienced a reversible grade 3 treatment-related adverse event.

The current study of CI-8993 in patients with solid tumors is currently enrolling at 0.6 mg/kg.

#### **Encouraging PK/PD Activity**

In the prior Janssen study, CI-8993 had demonstrated that, at low doses, a "sink effect" limited the amount of CI-8993 that could be detected in the circulation of patients. In the current Curis study, CI-8993 has shown non-linear increases in pharmacokinetic (PK) exposure at each dose level and exhibits saturation kinetics, indicating the potential to overcome this sink effect as we increase dose. These findings suggest the potential for broad bioavailability at higher dose levels. The pharmacodynamic (PD) effects of CI-8993 in patients observed to date suggest the possibility that CI-8993 can activate multiple anti-cancer immune mechanisms, including mechanisms that are not addressed by currently approved checkpoint inhibitors. Curis intends to further explore this PK/PD relationship at higher dose levels, as the study continues.

Curis expects to report expanded safety and tolerability data, along with initial PK, PD and anti-cancer data from the trial in the second half of 2022.

#### About CA-4948

CA-4948 is an IRAK4 kinase inhibitor and IRAK4 plays an essential role in the tolllike receptor (TLR) and interleukin-1 receptor (IL-1R) signaling pathways, which are frequently dysregulated in patients with AML and MDS. Third parties have recently discovered that the long form of IRAK4 (IRAK4-L) is oncogenic and preferentially expressed in over half of patients with AML and MDS. The overexpression of IRAK4-L is believed to be driven by a variety of factors, including specific spliceosome mutation such as SF3B1 and U2AF1. In addition to inhibiting IRAK4, CA-4948 was also designed to inhibit FLT3, a known oncologic driver, which may provide additional benefit in patients with AML and MDS.

#### About CI-8993

CI-8993 is a monoclonal IgG1 κ antibody with active Fc, designed to antagonize the V-domain Ig suppressor of T-cell activation (VISTA) signaling pathway. VISTA is a novel

negative checkpoint ligand expressed on myeloid cells and T cells that is homologous to PD-1/PD-L1. VISTA enhances T cell quiescence and myeloid derived immune suppressor cells (MDSCs). CI-8993 relieves negative regulation by hematopoietic cells and enhances protective anti-tumor immunity. Preclinically, VISTA monoclonal antibody treatment increased the number of tumor-specific T cells in the periphery, and enhanced the infiltration, proliferation and effector function of tumor-reactive T cells within the

tumor microenvironment (TME). VISTA blockade alters the suppressive feature of the TME by decreasing the presence of monocytic and granulocytic MDSCs and increasing the presence of activated dendritic cells (DCs) within the TME leading to enhanced T cell mediated immunity. VISTA monoclonal antibody administration as a monotherapy has been shown to suppress the growth of both transplantable and inducible melanoma in preclinical models. CI-8993 was originally developed as part of a license and

collaboration agreement between ImmuNext and Janssen Biotech, Inc (Janssen). In 2016, Janssen initiated clinical development of Cl-8993 in a Phase 1 study of Cl-8993 in patients with advanced solid tumors. The study enrolled 12 patients, in which one patient experienced dose-limiting side effects related to cytokine release syndrome. Afterwards, Janssen opted to close the study and ImmuNext regained control of the asset. Curis is engaged in a collaboration with ImmuNext for the development of Cl-8993.

## FDA LIFTS PARTIAL CLINICAL HOLD ON MDS AND AML MAGROLIMAB STUDIES

- U.S. Pivotal Studies to Restart Enrollment Immediately
- Decision Based on Review of the Comprehensive Safety Data from Each Trial

FOSTER CITY, CA – April 11, 2022 (BUSINESS WIRE). Gilead Sciences, Inc. (Nasdaq: GILD) today announced that the U.S. Food and Drug Administration (FDA) lifted the partial clinical hold placed on studies evaluating its investigational agent magrolimab in combination with azacitidine. The FDA removed the partial clinical hold after a review of the comprehensive safety data from each trial.

With today's decision from the FDA, enrollment in the United States can resume in the studies investigating magrolimab in combination with azacitidine for myelodysplastic syndrome (MDS) and acute myeloid leukemia (AML). Gilead, in close coordination with regulatory authorities, is planning to re-open enrollment in the magrolimab studies that were placed on a voluntary hold outside of the United States. The company is also working with the FDA regarding the remaining partial clinical hold affecting studies evaluating magrolimab in diffuse large B-cell lymphoma and multiple myeloma. The ongoing clinical studies

WITH TODAY'S DECISION

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(MDS) AND ACUTE
MYELOID LEUKEMIA (AML).

evaluating magrolimab in solid tumors were not subject to the clinical hold.

"Our confidence in the risk-benefit profile of magrolimab has been unwavering, and we continue to believe in the potential for this treatment to address the unmet medical needs faced by people living with MDS and AML," said Merdad Parsey, MD, PhD, Chief Medical Officer, Gilead Sciences. "This is a significant milestone for Gilead and, more importantly, for patients diagnosed with these cancers. We look forward to continuing our work developing magrolimab and advancing this potential cancer treatment option."

During the partial clinical hold, patients already enrolled in the affected Gilead

magrolimab studies, including the pivotal, Phase 3 ENHANCE study, continued receiving treatment. Prior to the trial hold, Gilead already met the pre-specified enrollment threshold required for the first interim analysis of the ENHANCE study. Based on this, Gilead is confident the readout for the first interim analysis remains on schedule for 2023.

Magrolimab was granted Breakthrough Therapy designation for the treatment of newly diagnosed MDS by the FDA in 2020. In addition to MDS and AML, magrolimab is being developed in several hematologic cancers and solid tumor malignancies. Magrolimab is an investigational product and is not approved by any regulatory authority for any use; its safety and efficacy have not been established.

#### **About Magrolimab**

Magrolimab is a potential, first-in-class investigational monoclonal antibody against CD47 and a macrophage checkpoint inhibitor that is designed to interfere with recognition of CD47 by the SIRPα receptor on macrophages, with the goal of blocking the "don't eat me" signal used by cancer cells to avoid being ingested by macrophages. Magrolimab is being developed in several hematologic cancers, including myelodysplastic syndrome (MDS), acute myeloid leukemia (AML) as well as solid tumor malignancies. More information about clinical trials with magrolimab is available at www.clinicaltrials.gov.

# KARYOPHARM RECEIVES ORPHAN DRUG DESIGNATION FROM FDA FOR ELTANEXOR FOR THE TREATMENT OF MYELODYSPLASTIC SYNDROMES

NEWTON, MA, Janurary 24, 2022 (PRNewswire). Karyopharm Therapeutics Inc. (Nasdaq: KPTI), a commercial-stage pharmaceutical company pioneering novel cancer therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for eltanexor, a novel oral, Selective Inhibitor of Nuclear Export (SINE) compound, for the treatment of myelodysplastic syndromes (MDS). 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 as a single-agent or in combination with approved and investigational agents in patients with several types of hematologic and solid tumor cancers (KCP-8602-801; NCT02649790). Previously, Karyopharm reported positive data from an investigatorsponsored Phase 1 study evaluating singleaaent eltanexor in patients hypomethylating agent (HMA)-refractory MDS, where eltanexor demonstrated a 53% overall response rate and median overall survival of 9.9 months. This compares favorably to historical survival of four to six months for HMA-refractory MDS patients.

Approximately 15,000 people are diagnosed with intermediate-to-high risk MDS each year in the U.S. 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.

ORPHAN DRUG DESIGNATION
BY THE FDA IS GRANTED TO
PROMOTE THE DEVELOPMENT
OF DRUGS THAT TARGET
CONDITIONS AFFECTING
200,000 OR FEWER U.S.
PATIENTS ANNUALLY AND
ARE EXPECTED TO PROVIDE A
SIGNIFICANT THERAPEUTIC
ADVANTAGE OVER
EXISTING TREATMENTS.

"We are pleased to receive the FDA's orphan drug designation for eltanexor in MDS and believe it reinforces eltanexor's potential to improve clinical outcomes for patients with HMA-refractory MDS," said Richard Paulson, President and Chief Executive Officer of Karyopharm. "We are focused on advancing our ongoing clinical trials and remain steadfast in our commitment to bringing this new treatment option to patients and their families."

Orphan drug designation by the FDA is granted to promote the development of drugs that target conditions affecting 200,000 or fewer U.S. patients annually and are expected to provide a significant therapeutic advantage over existing treatments. Orphan designation qualifies a company for certain incentives that apply across all stages of drug development, including the potential for seven years of market exclusivity following marketing approval, tax credits on qualified U.S. clinical trials, eligibility for orphan drug grants, and exemption from certain administrative fees.

#### About Eltanexor

Eltanexor (KPT-8602) is an investigational novel SINE compound that, like selinexor, 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. (Nasdag: 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 endometrial cancer, myelodysplastic syndromes and myelofibrosis. For more information about our people, science and pipeline, please visit www.karyopharm.com, and follow us on Twitter at @Karyopharm and LinkedIn.

#### **EVERY DAY IS A GIFT**

#### **JUNE EMMONS**

Grantsville, Maryland

My name is June and I have MDS. I was diagnosed in June of 2020. I live in the beautiful mountains of western Maryland in a ski/lake community with my husband of 46 years. We were just starting to enjoy our retirement when Covid arrived along with my cancer. I had not felt well for several months, fatigue, fevers, rash and over all not well. After seeing a couple doctors and my blood work looking off I was referred to a hematologist to look at my blood work. We have a very small hospital and cancer center forty five minutes away. My daughter went with me to the appointment. I had no idea how my life was about to change and not for the better. The doctor after looking at my blood work seemed to know what was wrong, MDS! My daughter and I asked questions for an hour but the doctor said I needed a bone marrow biopsy before he could be sure. The information we did get was awful.If indeed I did have MDS there was no cure and one to three years life expectancy. My whole world turned over. I was in disbelief. How could this be? I'm sixty one years old. I felt fear like I've never known before. I walked out in tears shaken to my core. My husband was waiting for me and I explained what was said the best I could but I had a thousand more questions. We cried together along with my two daughters. Now I had to somehow wait for the bone marrow biopsy. We alerted close





family and friends and prayers began. I couldn't wrap my head around it. They had to be wrong. Why me? I had cancer 36 years ago ( Hodgkin's disease). I was a survivor. How would I do this again? The day came for the biopsy. I refused drugs and just wanted to get it over with. The procedure was much easier than I had feared. Again I had to wait two weeks for the results. I just refused to believe it would be that bad. The day came to get the results. I didn't know how I would find the strength to get to the doctors office. My husband went with me. I just wanted to get it over with. The doctor came in and I said please tell me what I have. The answer, MDS with a chromosome three inversion and due to having chemo and radiation before my prognosis was poor. High risk with little or no response to chemo. NO! I wouldn't except this. I remember the doctor taking me aside and saying, June this is really bad. The rest of the day was a blur. Was I really going to die? I started doing research night and day. I read everything I could get my hands on. A lot of it was way over my head but I needed answers. Luckily a family friend was a cancer doctor and I reached out to him in desperation. I explained everything and sent my test results to him. He was my miracle. He explained things in a way I could understand and gave me encouragement to try treatment. He thought it could definitely work and was very

doable. So here I am twenty months later in remission and doing overall pretty well. I saw a total of four doctors. One was found through the MDS Foundation. They are an amazing resource for any MDS patient as well as a friend. They guided me to a wonderful specialist. The road has had many bumps and detours. Every day is a gift. I fight my fear daily and hang on to hope to get me through. I believe in miracles happening everyday, so why not me? I still read and research everyday. I know better treatments and a cure are close by. I am a survivor!!! Last I want to thank my devoted family especially my husband, two beautiful daughters, four amazing grandchildren, my sister and many dear friends. I love you all more than I can express!





#### **LESSONS LEARNED**

## CATHERINE MICALES McDonald, Pennsylvania

My 'journey' to my diagnosis began 31 October, 2020. It was Halloween night and within my neighborhood, we had a large bon fire down by our lake to celebrate the evening and give everyone a much needed break from Covid — 19 lockdown. The evening was quite brisk and with that, a few neighbors and I decided to walk down to the lake.

As we set out and with the air being brisk, I noticed that I was having a difficult time catching my breath. As we continued to walk, my breathing became increasingly labored and difficult to the point, that I had to return back to my home. At that point, I was convinced that I was having some type of cardiac event and after speaking to a dear loved one; decided the most prudent thing to do would be to go directly to the local ER in my area.

Initially, the ER doctors began to work me up for a pulmonary embolism which included, CT scans and tons of bloodwork inclusive of a CBC. The ER doc came into my room and asked if I had a history of being anemic to which I answered no. Her reply to me was, well this evening you are quite anemic in fact your hemoglobin is 5!

Being a Medical Technologist with a specialty in Hematology and knowing well, what my labs usually are I was convinced this was an error and asked if a new specimen could be drawn and repeated. In addition, and with a bit of irony; I also work in the pharmaceutical industry and have focused the majority of my career in hematologic malignancies; specifically, MDS and AML. And yes, I was one of those patients that knew far too much (this, said with a bit of a wry smile).

Repeat labs came back with pretty much the same results. I was speechless to be honest! I asked the ER doctor if I could see the entire CBC results to which she obliged. It was then, that I knew that there was something much more ominous going on and that I likely



had either MDS or AML based on the results. I was completely petrified and without words.

Subsequently, I was admitted to hospital for blood transfusions and ultimately referred to a Hematologist/Oncologist at West Penn Hospital in Pittsburgh, PA. From there, the confirmation of diagnosis began with bone marrow biopsies, blood work, mutational analysis, etc, etc, etc.

On 29 December, 2020 I received a call from my Heme Onc with the news that based on all results and confirmation from the pathology read, I had intermediate to high

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risk MDS-EB1 with NPM1 and DNMT3A mutations. The course of treatment was discussed and I started Decitabine and Venetoclax in January of 2021 with the intent, to promote a complete response so that I could then, move into allogenic stem cell transplant.

I was blessed and within a span of two months, I achieved a partial response (labs were relatively normal and the frequency, of the mutations dropped significantly). From there, we moved into the pre transplant process and by March, 2021 I presented to hospital to begin conditioning chemotherapy.

As I mentioned above, I am one of those patients' that know a little too much as it relates to my disease and treatment. I will be completely honest; it was at this time that I cursed knowing what I did as ignorance truly can be bliss. I decided however; to try and look at the situation in a different light and recognized that my knowledge of the disease, the treatment process, etc would (not could but would) be an advantage. In particular, because I was able to explain the complexities of the disease and treatment process to my family, loved ones and friends... the 'largeness' became more manageable and less stressful for everyone....knowledge truly is power!

Those of you that have gone thru transplant, are familiar with hospital jail. The forty some days that you are essentially locked down on the transplant wing dealing with the myriad of side effects from conditioning therapy. I will say, that while this time was certainly not enjoyable where it not, for the support of my loved ones, family and friends I certainly would have not done as well as I did.

In addition, the nurses, my physicians, nurse practitioners, nursing assistants and fellow patients were amazing sources of light inclusive of the many discussions we had about the latest and greatest Netflix series. Truly though, I cannot thank enough all of the wonderful healthcare providers that took care of me during my 1 st stem cell transplant. They became friends and certainly were there for me.

All considered and certainly, in comparison to some of the other patients that were on the floor with me during my 1st transplant, I did remarkably well. The biggest challenges for me were mucositis (which started around day +4 and the GI issues which started at the same time).

By the end of day +38 it was evident, that I had failed to engraft and much of that was attributed to the fact that the donor stem cells were frozen (because of Covid-19) and very likely that plus the preservatives used impacted the viability of the donor cells.

I was ultimately discharged from hospital on day +39 at a beautiful time of the year. It was in fact, perfect for what I was going thru on a physical level. Spring...rebirth and indeed, my body was fighting hard to regain control and come back to normal. As time went on, we remained vigilant checking chimerisms for donor engraftment.

The great news is, I did engraft with my own cells and by July, 2021 my counts were back to normal! Unfortunately though, chimerisms showed no evidence of donor engraftment. Likewise, my DNMT3A mutation was rising and the decision was to revisit a 2nd transplant which ultimately, occurred in September, 2021.

During the Spring and Summer months of July, 2021 I felt amazing! I was able to travel to my home state of NJ and visit family, spend some time on the Jersey shore, eat some amazing seafood, bagels and pizza. I looked fantastic and felt incredible and while, the knowledge of the disease possibly returning existed; I focused on the beauty of each day. Something quite frankly that this disease taught me more than ever to do...to live in the moment and celebrate the small wonderful things that exist in this world. What a beautiful lesson indeed.

I was able to return to work as well full time for a brief period and prior to heading back into transplant #2. That too, was honestly a blessing as I was able to re engage my brain and feel and appreciate 'normalcy' which I greatly missed. As September, 2021 approached, I was re charged and ready to head back in and fundamentally; kick MDS squarely in the pants!!!

This time around, my donor and I matched perfectly 12/12 and I was convinced that this time transplant would be a success and I would be celebrating in a few short months. And so, I once more checked into transplant for round #2 on 2 September, 2021.

My first day on the floor I had the absolute honor to meet a woman with whom, I shared the same diagnosis and who also, failed to donor engraft with her 1st transplant. This angel would become my daily interaction on the floor. We laughed together, cried together, complained together, walked together, shared stories about our families and loved ones, pulled pranks on one another. We even received our stem cells on the same day (9 September, 2021)... truly Ellen was my transplant angel here on earth.

## LESSONS THAT I LEARNED FROM TRANSPLANT:

TRULY APPRECIATE ALL THAT
YOU HAVE IN YOUR LIFE AND
IN PARTICULAR, THE PEOPLE
THAT ARE IN YOUR 'TRIBE'...

...BY ALLOWING AND
EMBRACING HELP I WAS
PROVIDING THOSE IN MY LIFE
THE AVENUES THEY NEEDED TO
HELP, TO DO SOMETHING.

Second transplant for me, was a bit different primarily because of a different conditioning therapy. My hair fell out at about day +10 and it didn't come out just a little bit... it came out in large patches and all at once. Keeping with the attitude that laughter is the best medicine....I walked over to Ellen's room and told her the whole experience made me want to pull my hair out and so, being overly animated I proceeded to grab a fistful of hair and yanked it out....we laughed until we cried and laughed some more.

That is the thing about a disease like MDS or AML; it is such an emotional roller coaster. Side effects in and of themselves are wicked, the reality of being away from your home, your loved ones, your fur babies, worrying about whether your marrow will recover, whether your disease will return, etc. It is an unbelievably wicked ride, however; if you are fortunate enough to have an amazing tribe of people around you...the ride can become a life changing journey. And indeed, mine was.

With regards to the outcome of transplant #2 for me, once more I failed to engraft my donor cells. After a lot of discussion and consultations by my physicians with physicians at some of the larger academic institutions in the US; it was determined that my marrow was too damaged from disease.

At this time, I am struggling still at day +130 to engraft my own cells and remain transfusion dependent. With regards to disease and mutation status, we are in a watch and wait scenario with the plan, to address any return of disease via chemotherapy as needed. I am also, very happy to report that I will be returning to work in mid-March, 2022!

I'm often asked, how did you prepare for transplant and what are some of the things you should bring with you to the hospital? I'm hardly ever asked, what lessons did you learn from transplant and how did it change you? These are two items that I would like to share as for me; the lessons and how transplant changed me are the crux of my journey.

Lessons that I learned from transplant are to truly appreciate all that you have in your life and in particular, the people that are in your 'tribe'...your family, your loved ones, your friends. They are terrified for us and simply wish to do something/anything to help. For me, accepting help has always been a challenge as I didn't want to burden anyone. I learned; however, by allowing and embracing help I was providing those in my life the avenues they needed to help, to do something. And so, please....don't be afraid to ask for help and accept help gracefully. It is a beautiful act of love and allows us to share our journeys with those who care for us.

How did transplant change me? This again, was huge for me. As I mentioned at the start. My professional career is based in the pharmaceutical industry and specifically, in Clinical Operations Research and Development. Essentially, what I do for a living is oversee and effect clinical trials in hematologic malignancies.

The 'exposure' that I have thru my role/job is the opportunity to interact with renowned physicians, access to the most up to date 'data' from clinical trials, endless learnings/teachings as it relates to these diseases. Most importantly though, access to patient data in particular data that is collected thru the auspices of a clinical trial. I have participated in patient advocacy programs and have always, been 'sensitive' to the plight of any patient fighting a devastating disease.

This experience however and, being now a patient, myself opened my eyes to a completely different element of 'fighting'. I

can now very proudly say, I truly understand what the meaning of a warrior is because I had the distinct pleasure of meeting so very many of them personally and walking literally, together with them on our collective journey.

The disease itself is one thing to deal with but it's all that comes with the disease that together; make you really embrace reality and fear. Side effects, will the disease come back, will my loved ones be ok, will my fur babies be ok, what does the future hold, how much more treatment will I allow myself to go thru, what secondary effects from treatment will I face, why is my skin color so off, how will the world that does not know my story embrace my new look and me? The list goes on and on and these, are the questions and so many more that every patient faces.

Certainly, there are days when I find myself going down the proverbial rabbit hole. I became dependent on the serenity prayer to keep myself focused and not, let myself go too far down the hole or remain there too long. The reality is this, we are so amazingly blessed and surrounded by so many people that love and care for us. Random acts of kindness from strangers... a simple smile, a simple compliment can be enough to lift your spirits and pull you back to the mindset of 'Yes, I am here, I am alive and I can and will still be able to contribute to life'.

Live your life loud and proud. Pay forward as much as you possibly can and understand always, that we all are fighting something...it may be obvious then again, it may not. But simple human kindness is truly one of the greatest blessings we must give to others.

Thank you for enduring my story and likewise the opportunity afforded to me by Audrey Hassan of the MDS Foundation.

I would like to end this with a special dedication to my transplant angel Ellen who is now I am certain, actively filling her charge in the heavens above. Rest in peace my dear brave friend.



#### MY MDS, MY BODY, MY LIFE

GARY MILLER, DHSC
AND MDS PATIENT
Bedford Lakes, New Jersey

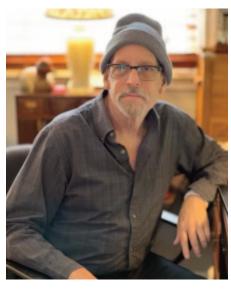
#### **BACKGROUND**

When first approached with a request to write something for the MDS Foundation's Newsletter, I acted on my natural default setting of "yes, of course — anything that I can do, I will do, if it helps other MDS patients or advances scientific understanding of this somewhat obscure condition".

But it wasn't long before second thoughts began to creep in; not out of any reluctance to help but rather the fact that my MDS disease and treatment experiences really haven't been all that remarkable. Yes, I have the condition and yes, I've received both chemotherapy and a bone marrow transplant and yes, there have been (and are) side effects. But all this was not only anticipated but was made manageable through knowledge, understanding and choosing to be an active participant in my care.

I've read several personal accounts written by other MDS patients that have been printed in previous MDS Foundation Newsletters and I fast-learned that nothing that I could craft could remotely compare to their inspirational eloquence. They were all, in their own way, quite moving and I saw myself in many of the stories. It was clear that if I was to compose something meaningful here, I would need to take a different direction.

And that's when a notion began to form; there might actually be something poignant that I could offer after all and it came from the above statement that side effects were anticipated (with "anticipated" being the operative word). This one simple word represents the cornerstone of the approach that I took in dealing with MDS. Being able to anticipate certainly has made and is making this experience vastly easier as I continue on my pathway of progress. Perhaps others who are about to go through their own MDS voyage might gain something from what I can impart.



In advance, please, I beg forgiveness if I come off as preachy or as a know-it-all. I make every assurance that I am no minister and I most certainly do not know everything about this disease; not even close. However, I do clearly recognize what has been of invaluable importance to me in my recovery.

So, at the risk of displaying unforgivable hubris and perhaps no small measure of sanctimony, I'd like to offer a few pointers that might put some newer MDS patients at ease as they approach the myriad of tests, treatments, doctor visits and medication regimens that lie in store for them in their movement forward in addressing their

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medical situation.

It's noteworthy to mention that this is the third rewrite of this article. The ones that preceded seemed a bit heavy-handed and overly assertive so as to appear aggressive. That is not at all the image that I wish to convey. Rather, I simply would like to communicate to all new MDS patients that there is real hope and that it starts with how one approaches the management of their disease.

#### **UNDERSTANDING MDS**

Knowledge is Power. We have all heard this adage; and it is, in almost all instances, a truism. If I might be so bold, I'd add a few words to this saying so that it reads "Knowledge is Power and Power is Control" Never has this been more apropos than when applied to MDS. Learning about MDS leads to empowerment. And from that empowerment comes an assurance that allows one to be able to control their challenging situation. I was far better prepared for what lay in store because much of the mystery and befuddlement surrounding MDS had been erased. I could then clearly see my opponent. I understood him and was prepared to begin battle.

For most people receiving a diagnosis of cancer, there are natural reactions that are often predictable. Included in these are shock, or denial, or a feeling of dread or helplessness. Frequently, depression accompanies. None of these should be considered insignificant or frivolous and summarily ignored. Each is tremendously important and warrants discussion with your chosen care provider.

All of these emotions are certainly understandable; some to be expected. So much of what we have witnessed with and heard about cancer has been horrific: lots of suffering followed by a poor outcome.

Cancer certainly can take that route but with early detection and the remarkable progress made with new and effective treatment options, cancer is certainly not the negative fait accompli it once was. The more one looks into recent oncologic advancements, the more real hope can be found for a positive outcome. I would take this further to say that it goes

beyond just hope and becomes a realistic expectation for a cancer-free future.

When a diagnosis of MDS is received, one of two things is likely to occur. The patient might choose to assume a passive approach and follow the treatment recommendations of qualified medical staff assigned to them. This is quite acceptable and works well for many individuals.

Some patients, though, see it differently. They employ a more assertive and hands on approach. I chose this method because I felt a driving need to not only be prepared for what could happen, I found it necessary to become an active participant in my recovery. Being armed with knowledge and understanding of MDS provided the strength, confidence and determination that I would require to face all that was about to come.

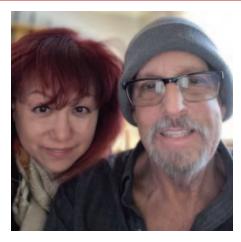
#### **TIPS FOR LEARNING**

The assertive approach starts with education; proper education. Firstly, I'd advise one to not place much stock in anecdotal experiences of family, friends or bloggers. Well meaning as they are, the information is often dated, seldom conveyed accurately and usually involves circumstances that are not germane to your case.

Secondly, I'd suggest that you attempt to discard old preconceived notions that you might have about cancer and begin a search for current information from reputable sources whose only interest is to freely provide knowledge, absent any expectation of gaining something in return.

The task of acquiring knowledge that leads to an understanding of MDS and the various treatment options that exist can be daunting; even for someone familiar with things medical. I humbly pass along to anyone interested, a few effective strategies that I employed.

1) If, in doing the inevitable internet search, a site pops up with the word "Ad" in the front, understand it for what it is. It's an advertisement by some entity with a vested interest in impressing upon you their product. The site could possibly provide some general and cursory information but it's ultimately designed to guide the reader toward a



Me and my wife, Wen, who is my primary home caregiver.

particular philosophy or to buy into their merchandise. I did not find these sites of much value and would suggest that you not spend much time with them

2) It's advisable to limit your search to postings that go back no more than 5 years (unless of course you are researching the history of MDS, which is a fascinating study in and of itself). Scientific understanding of MDS has grown exponentially in recent years and this has led to remarkable discoveries and vastly more effective treatment options. The more recent the post, the more topical the content.

3) You will do well to stick to reading that which is offered by well established sources known for their medical excellence and philanthropy; like (but not exclusive to) the Mayo Clinic, the American Cancer Society, the MDS Foundation, the CDC and many reputable teaching institutions. There is an abundance of good information readily available to anyone interested. All one need do is to make some simple internet inquiries.

Parenthetically, kindly indulge me for just a brief moment as I say a word or two about MDS Foundation support. They have put together some fantastic information that I consider a must read for anyone with MDS. A patient who is armed with what they offer will be given a true and accurate understanding of their condition. This will prove comforting.

Moreover and very importantly, it's the well-informed patient who will find him or herself in good stead with both their outpatient and in-hospital care teams. These teams will surely pick up on how current you are with MDS and how interested you are with your treatment. They will automatically come to view you less as a patient to be treated and more as an individual to be consulted. Additionally, you will be seen as an equal member of the care team. Hence, you will always be kept informed of what's going on and why. And because you will be considered a team member, your voice will be heard and all will be open to your suggestions. In my case, I had some in-hospital BMT complications. It has been enormously reassuring to see how we all worked together as a unified team focused on addressing whatever issues presented. This continues with post-discharge concerns that have arisen.

4) While it's difficult, nay near-impossible, to not get hung up on things like "life expectancy" and "survival rates", please try to resist, These are complicated projections and understanding what they actually mean can be a challenge. Therefore, it is easy for one to draw inaccurate conclusions. The life expectancy and survival rates that we read for MDS situations are a compilation of several confusing variables thus, they can be dangerously misleading. (example: the internet sites have my survival rate with a BMT as 4 - 8 years when in fact, I can actually look forward to 20+ healthy and quality years). It is far better to simply discuss this with your Dr. He/she will gladly explain your particular situation as it relates to your future.

## YOUR CARE PROVIDERS - OUTPATIENT

Let's discuss the tremendously important area of selecting an outpatient oncologist and accompanying treatment team.

I pause here to state something that I believe to be critical. It is a precept that has already been noted and will resonate throughout this article. Please remember, always, that you are a member of Equal Standing within your treatment team. Make no mistake, your thoughts and concerns are every bit as important as anything offered by a Dr, PA, NP, technician or nurse. Their contributions stem

from years of education; yours are experiential. This is your treatment. It's your life.

That said, let's examine what's important to consider when choosing an oncologist. As with any care provider that you would select, you must be comfortable with this person. Mutual trust between you will need to be established. This may sound obvious but sometimes the Dr/Patient relationship can be lopsided and such could well lead to frustration and dissatisfaction by either party. Refreshingly, there seems to have been a kind of mindset shift in recent years whereby medical care providers are displaying more compassion and empathy for their patients than in generations prior. They are now less likely to talk at patients as they are to talk with them.

The availability of MDS-knowledgeable oncologists varies considerably depending on what area of the Country you live and the selection pool can be limited in some places. All you can do is your best to find someone with whom you can be comfortable.

A good oncologist will put you and your concerns first, above all else, and will welcome your questions no matter how simple or uninformed or even inane they might seem. He/she will take time with you in all cases to address your concerns thoroughly such that you do not feel like just a number being rushed through a process.

In my case, I have a fantastic local oncologist and treatment team. They handled the chemotherapy. This physician and his team came to me via a selection process that I undertook. This exercise proved to be surprisingly easy because the parameters were simple; who in my area had not only a distinguished history of treating MDS but also possessed a solid overall reputation of excellence. I checked several oncologists for their education, their specialty studies and any patient reviews that I could find.

When satisfied with the credentials of a likely physician, next comes the interview process. The term "interview process" is used purposely as it is just that; an audition of sorts wherein both the physician and patient begin to learn about each other. There should be no mistake that you are hiring this person to care for you as you go through the most serious of

health situations that you may ever encounter. Should there be any sort of uncomfortable feelings, try expressing them in the initial meeting. Hopefully, these can be allayed straight away. However, and if the feelings persist, it might be wise to look for another provider. But likely, this will not occur and you two will hit it off famously and begin a journey together based on mutual trust and respect.

## YOUR CARE PROVIDERS - INPATIENT

Sometimes, the MDS patient will require hospital care. It could be for several reasons but for me, it was for a bone marrow transplant (BMT). Therefore and while I focus on BMT here, the concepts that follow are not BMT-specific and apply to in-hospital care for most other situations.

A BMT is an involved process consisting of many phases. It is performed in-hospital and requires that the recipient be admitted for a few weeks.

Again, this procedure may seem intimidating so, it's best to have already researched BMT in anticipation of it being a possible eventuality. Again, knowledge can demystify even the most incomprehensible and frightening of situations.

Things are quite different when it comes to inpatient treatment in the sense that there are a lot of moving parts going fast and you will have little say as to who will be providing your care. Try not to be overwhelmed but go ahead and employ, where possible, the same principles used for gaining general MDS and BMT information that you did for outpatient care and apply them to in-hospital treatment.

Establishing yourself as a knowledgeable care team member within the hospital is especially important. Now is when all the information and understanding about MDS and BMT that you have acquired will reap valuable dividends. As with outpatient staff, hospital personnel will especially respect that you are on top of your condition. They will treat you more as an equal team member when they see that you are both proactive with your care and up to speed with MDS/BMT. This is not to say that you need to question everything that they try to do. It is to

say that by showing an active interest in what's happening, you will be kept in the loop to the degree that you wish. Should you question something, know you have every right and are encouraged to ask anything. I always took the position that hospital staff were doing things for me, not to me.

#### **POST DISCHARGE**

After a BMT, there will be a lengthy and monotonous period of continued treatment, testing, medication and Dr visits. And while I'm tempted to highlight these, I fear that I've already droned on too long in my verbosity to delve into this to any great degree. Also, such would risk redundancy. Suffice it to say that one can approach post discharge activity similarly as they did with the acquiring of outpatient and in-patient treatment education that preceded.

By now, most MDS patients will be quite experienced in and familiar with how things go. This can provide real and lasting assurance and confidence to guide them along as empowered and self-directing consumers of care.

#### CONCLUSIONS

I hope that some of the above will help the newer MDS patient to navigate the important treatment considerations that their condition may require.

I hope that the newer MDS patient is less fearful and intimidated about his/her upcoming care.

I hope that the newer MDS patient sees the hope and promise that modern treatment offers.

I hope that the newer MDS patient feels more empowered and is able to take an active role in his/her care.

And I mostly hope that the newer MDS patient begins by viewing all of this as it's **My MDS**, **My Body**, **My Life**.

"YOU ARE BRAVER THAN
YOU BELIEVE, STRONGER
THAN YOU SEEM, AND SMARTER
THAN YOU THINK"
A. A. MILNE

#### FRIEND TO FRIEND...

#### **RICK JOHNSON**

Demotte, Indiana

I thought I would speak to you as a friend and as a friend I promise to tell only the truth. So as a friend, I should tell you a little about myself. I was diagnosed with MDS about 5 years ago and tried three different chemo's without success. Not being a transplant candidate, my doctor said we might try IDHIFA (enasidenib) but it would be around 2 years to get approval. I was lucky Trump signed into law the Right to Try Act and I had my medication in my hand in 2 weeks. Again lucky the medicine agreed with me and has helped me immensely. Months later, weak as a kitten, I went to see my brother in North Carolina, and we decided to go for a hike as we had when we were kids. I love hiking and in 2019 I hiked 1,980 miles and have the fit bit to prove it! In 2020, I had Covid and the pneumonia. Now on the mend, my plan is to hike 2,000 miles in 2022.

When I was growing up my mother was never more than arm's length from a good book. She told me that if you can read, you can do anything. So as you have stayed with me this far I have to believe you are capable of doing anything.

So as a friend I will tell you about some of the things that I have observed.

MDS is like a brick wall: it's ...HARD!! It is unmoved and without compassion. But I have observed a curious thing about walls. You can go over them , under them, around them, and through them!

Now this is a part that I would ask you to pay extra special attention to because you could miss it.

The most precious people in your world will RAISE THEIR HANDS AND LIFT YOU UP!!! They will be coming to you from places you would not expect.

I would illustrate this with something that happened to me. I was having a no good, horrible, very bad day in ICU. The man who empties the trash saw I needed a friend and talked to me, and after a while I felt better about everything. He raised his hand.

My case was difficult to figure out as I had a lot going on. Every last person at the University of Chicago raised their hands and lifted me up. I am in such awe of them and I am grateful!

I would suggest to you that gratitude is a strength that you can rely on. Your gratitude will bring others who will raise their hands to lift you up. After a while there will be legions, as my experience has been.

I am humbled by the events that have led me here.

So, my newfound friend ....what are you going to do about that brick wall? Go over, under, around or .... OBLITERATE !!!!!!!!

That said, I would bless you with my favorite old Native American blessing:

"WALK IN BEAUTY"







#### **AML RESOURCES**



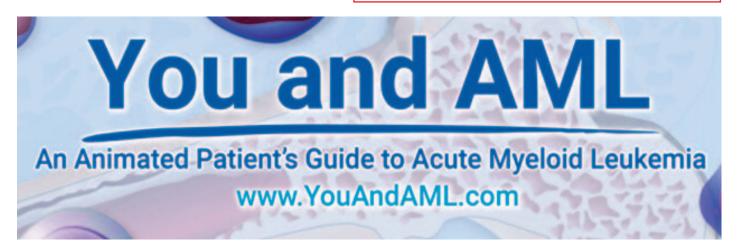
This resource is intended for patients with acute myeloid leukemia (AML). You will find expert advice about AML, AML with myelodysplasia-related changes (AML-MRC) and treatment-related AML (tAML) to help you discuss key issues with your healthcare provider and make important decisions related to management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

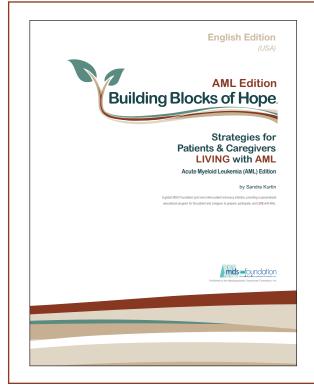
#### "YOU AND AML" CONTAINS 4 LEARNING MODULES:

- 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<sup>®</sup>, 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

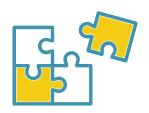
Chapter 4: The MDS Foundation



**Navigating Secondary** Acute Myeloid Leukemia

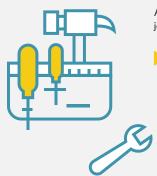
Do you or a loved one have myelodysplastic syndromes or secondary acute myeloid leukemia and are looking for resources or ways to connect with others on your journey?





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.

#### 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.3

#### **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. 4



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. 4

Visit FindTheRightFit-sAML.com to learn more about sAML subtypes and treatment options.

 $Visit\ Find The Right\ Fit-s\ AML. com\ to\ learn\ how\ MDS\ can\ progress\ to\ s\ AML.$ 

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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## ACUTE **MYELOID EUKEMIA**

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











#### **KNOW AML**

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

## SIGNS **SYMPTOMS**

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

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

- **✓** WEIGHT LOSS

- ✓ BLEEDING/BRUISING MORE EASILY



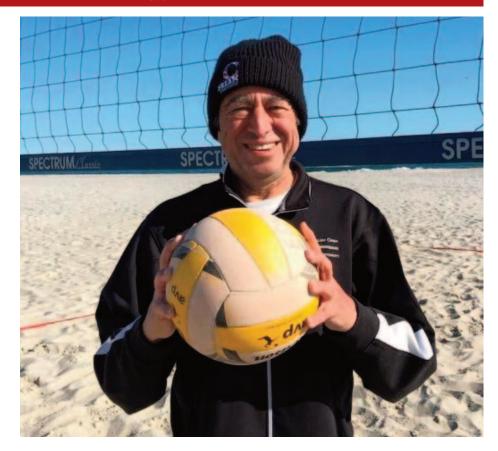
#### MY MDS ADVENTURE

#### **MARK TOPAZ**

Long Beach, New York

My name is Mark Topaz and my MDS adventure started in December of 2018. When I went for my 3-month checkup, my GP told me that I had a low white blood cell count and referred me to a hematologist. A bone marrow biopsy was performed. Apparently there are 3 indicators for MDS but they were only able to extract 2, of which one looked like I had MDS and the other did not. So I was not diagnosed and it was suggested that I receive B12 shots once a month. In early March, when I went for my shot, I was told that my red blood count was low and that I had to get a transfusion immediately. I then made an appointment to see another hematologist in mid March. It was then that I got the MDS diagnosis. But this doctor did not seem overly concerned and made another appointment for me to come back in late May. I then began doing research, which is when I found Audrey at the MDS Foundation. I contacted her and found her to be extremely helpful. She told me that the doctor I had seen was unknown to the Foundation and she recommended that I see one of their doctors of excellence in the field. I agreed and she was able to get me an expedited appointment to see Dr. Steven Allen at Northwell Hospital.

HAD I NOT CONTACTED
THE MDS FOUNDATION
AND GOTTEN THEIR HELP,
I PROBABLY WOULDN'T BE
HERE WRITING THIS NOW.
AND IF I WAS HERE, I DOUBT
I WOULD BE AS GOOD
AS NEW, AS I AM NOW.



This was in mid-April and after a very thorough examination, I was told that my MDS had turned to acute leukemia and that I had to go into the hospital right away.

This was quite a blow. A little background on myself. I was 62 years old at the time and I had never been sick and never had a hospital stay in my life. The only time I had ever even been in the emergency room was for a kidney stone and a dislocated pinky. After hastily making arrangements for friends to take care of my cats and tropical fish, I was admitted to the hospital the next day. The first round of chemotherapy didn't put me in remission but the second round did. They then began testing me to see if I was a candidate for a bone marrow transplant to cure the MDS, because without getting to the source of the problem, it would just bring the leukemia back. I got out of the hospital after 2 months and then had 2 rounds of consolidation chemotherapy. Because I don't have any family other than a sister in another country, I had to get a donor from the registry and it was

an 8 of 10 match. I returned to the hospital in early October and after a week I received my new cells. Except for one day where my fever spiked, caused by what they call the Hapro storm, I didn't really feel badly after the transplant.

It's now a little over a year since I went through this ordeal. I would say I'm 95% back to how I was before. I either walk or bike ride a few miles every day, play tennis and beach volleyball, go out dancing at night. My blood counts are good and my tests show 100% donor cells. My legs get a little sore but that may be as much from the long period of inactivity as anything else. I hate to think about what would have happened without Audrey and the MDS Foundation helping me. Had I not contacted them and gotten their help, I probably wouldn't be here writing this now and if I was here, I doubt I would be as good as new, as I am now. Audrey, Dr. Allen and Dr. Bayer, who's my BMT doctor, saved my life and I was very fortunate to have found the MDS Foundation.

#### AML CAREGIVER STORY

## STAN STANEK HAS STAN-I-MA!

#### **SUE STANEK**

#### Chanhassen, Minnesota

Almost out of the blue, on 12/1/16 my 60-year-old healthy-looking husband, Stan, was diagnosed with Acute Myeloid Leukemia (AML). He was told he not only needed intense chemo to attempt to get the cancer from spreading like wildfire, he would also need a bone marrow transplant to attempt to survive.

The next 7 months were filled with chemo. The first rounds were so intense he needed to be hospitalized 55 days for the treatment. He achieved remission for a short period of time, but the cancer returned. He then received a different type of outpatient chemo for 3 months, which unfortunately did not work. Along the way, due to his compromised immune system, he contracted the norovirus and also had emergency gall bladder removal surgery. It was a really tough time in our life, always hoping it would get better.

As we were coming to the realization the chemo was not working, we found out about a clinical trial at MD Anderson Cancer Center in Houston TX. The trial used a new approach that was targeted at a specific gene mutation which is present in less than 20% of AML patients. If he met the trial's specific criteria, and if he was randomized into the trial, we were told this would be his best bet to achieve remission. Against some pretty steep odds, he did meet the criteria, and was randomized into the trial in July of 2017. The clinical trial protocol included a daily med that we coined: "the magic pill." While Stan continued to take the drug, his brother Rob (a perfect match donor) had his donor cells harvested and frozen, hoping Stan would eventually need them. After 6 months (and 18 trips from our home in Mpls, MN to Houston, TX), he achieved complete remission and was evaluated as ready-for-transplant in February of 2018 at the Mayo Clinic in Rochester, MN. A transplant requirement was to live within a few miles of Mayo Clinic for 100



## ...WE LOVE OUR LIFE, AND APPRECIATE IT MORE DEEPLY THAN WE EVER WOULD HAVE OTHERWISE.

days. We packed our bags and took up temporary residence in Rochester, 100 miles from our home.

Stan received his bone marrow transplant on Feb 23, 2018. The doctors were optimistic & realistic, sharing that the success rate was a little better than 50%. We hunkered down in our local residence and ventured to the hospital each day for testing & monitoring. A few unplanned adventures included a stay in the hospital for a week due to complications, and a scare that sent us to the ER. It was a very challenging time, taking a huge toll on his body. We told the doctors: "this ain't for sissies!" (they completely agreed)

We were beyond-thrilled-and-almostbelief when Stan's post-transplant 100-day bone marrow biopsy showed no sign of leukemia. He has had yearly biopsies since and remains in complete remission. While Stan is still experiencing transplant-related issues and is currently being treated for chronic GVHD (graft vs. host disease), we love our life, and appreciate it more deeply than we ever would have otherwise.

## A few of the remarkable things that have kept Stan thriving:

1) He has chosen to walk 10,000+ steps daily. Even when he was in the hospital he lapped the 200-step "track" around the Oncology floor. During his transplant journey, he walked the hallways of Mayo. Now, during our Minnesota winters he bundles up to walk outside or does laps at a local mall. It was during his hospital stay one of the health care providers commented on Stan's walking regimen, saying "that man's got Stan-i-ma!" It stuck, and Stanima became his nickname.

2) Without denying the very serious prognosis of the situation, Stan decided to focus on being positive & hopeful. While he had never done this before, from day-one of diagnosis he started tracking his daily gratitudes on a calendar. These calendars now wallpaper his

#### AML CAREGIVER STORY

home office. Tracking gratitudes has helped remind him, and us, of making each day well-lived.

3) Stan has grown in his faith. We decided to pray out loud, together, each night. We had done this earlier in our life, but it had dwindled over the years. In our prayers we count our blessings, pray for others, and make our requests. About 6 months into the journey, we had a prayer service at our church for Stan, anticipating a small circle of about 20 people. We were overwhelmed when 165+ friends and family poured into the service, and gave us the most important support they could offer.

4) Stan shares his story. Through the website CaringBridge, we have been able to keep our friends and family posted on what is happening. Over 1200 different people have visited Stan's site, with 50 to 300 reading our journal updates, and many posting back with words of encouragement. What an amazing avenue for support – both ways. Also, we look for ways to support others on life-threatening health journeys, offering our story as hope & encouragement. (We also recognize how important it is to be sensitive &



compassionate to similar journeys with different outcomes.)

5) Since diagnosis, Stan chooses a theme for his life each year. In 2017 Stan chose RESURGENCE. In 2018: THRIVE. 2019: STRONGER. 2020: ADVENTURE. 2021: VITALITY. 2022 will be determined

soon! These watchwords are reminders of what he wants to intentionally choose for the second chance at life he has been given. While we wish cancer had never entered our lives, we can honestly say we have, in many ways, become better because of it. In Stan's words: let's choose to have AML refine us, not define us.



WHILE WE WISH CANCER
HAD NEVER ENTERED OUR
LIVES, WE CAN HONESTLY
SAY WE HAVE, IN MANY
WAYS, BECOME BETTER
BECAUSE OF IT.
IN STAN'S WORDS:
LET'S CHOOSE TO HAVE AML
REFINE US, NOT DEFINE US.

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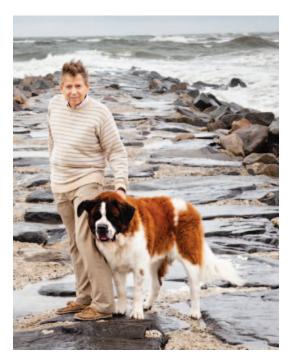
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We are offering an opportunity to memorialize your loved one (at no cost) on the MDS Foundation website.

If this is something you may wish to consider, please visit our Memory Wall.

www.mds-foundation.org/memory-wall

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

## Do you or someone you know have newly diagnosed Higher-Risk Myelodysplastic Syndrome (MDS)?



Consider enrolling in the VERONA study—a clinical research study evaluating venetoclax in combination with azacitidine, versus azacitidine alone. The study is evaluating the effect of these treatments on Higher-Risk Myelodysplastic Syndrome (MDS).

#### Participants must meet the following criteria:

- 18 years of age or older
- Newly diagnosed with Intermediate, High-Risk or Very High-Risk (Higher-Risk) MDS
- Have not received prior treatment for MDS
- Are ineligible for a stem cell transplant OR are eligible for a stem cell transplant, but have not yet identified a donor or arranged for the transplant

If you meet these criteria and are interested in participating, please contact your doctor to discuss the VERONA study and your eligibility.

For more information, visit ClinicalTrials.gov and search NCT04401748.

Venetoclax is an investigational drug that is not approved by the FDA or other global health authorities in MDS. Safety and efficacy have not been established in MDS.

## SELECT-MDS-1 Trial for Patients with Higher-Risk MDS Now Open at Sites Across the U.S.

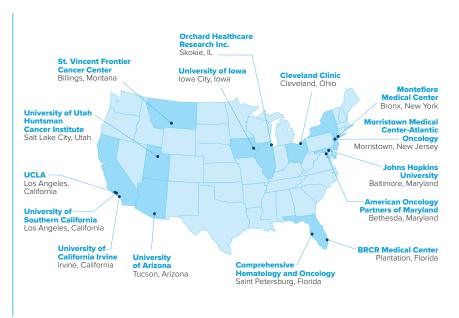
Syros Pharmaceuticals, a leader in the development of medicines that control the expression of genes, is continuing to enroll patients in the SELECT-MDS-1 Phase 3 trial across multiple open sites in the U.S.

The SELECT-MDS-1 trial is evaluating the clinical activity of tamibarotene in combination with azacitidine in patients who are newly diagnosed with RARA-positive higher-risk myelodysplastic syndrome (HR-MDS). Approximately 30% of HR-MDS patients are RARA-positive due to overexpression of the RARA gene.

Tamibarotene is a highly selective, potent oral RARa agonist and was recently granted orphan drug designation by the U.S. Food and Drug Administration (FDA) for the treatment of MDS.

Ask your doctor about finding out if you are RARA-positive and are eligible to participate in the SELECT-MDS-1 Phase 3 trial of Syros' oral investigational agent tamibarotene.





<u>ClinicalTrials.gov - search NCT04797780</u>. Tamibarotene is an investigational agent and has not been approved by the FDA as a treatment for any indication.

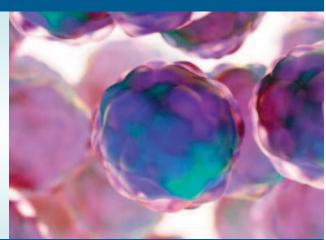


# IVOSIDENIB (AG-120) **NEW CLINICAL TRIAL NOW ENROLLING**

## 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 (NCT03503409).



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



Please contact Servier Medical Information: E-mail: medinfoUS@servier.com Phone: 1-800-807-6124.

Have you or someone you know been diagnosed with Myelodysplastic Syndrome (MDS) or Acute Myeloid Leukemia (AML)?

Consider enrolling in the TakeAim Leukemia trial - a clinical research study evaluating emavusertib alone or in combination with azacitidine or venetoclax. The study is evaluating the effects of these treatment on Higher-Risk Myelodysplastic Syndrome (hrMDS) or Acute Myeloid Leukemia (AML).

navusertib is an investigational drug that is not approved by the FDA or other global ealth authorities in AML or MDS. Safety and efficacy have not been established.





Contact your doctor to discuss participating in this clinical trial. For more information about emavusertib (CA-4948) visit ClinicalTrials.gov and search (NCT04278768).

## Lower Risk MDS Clinical Trial Now Enrolling HEMANT



A clinical study to evaluate the safety and efficacy of an oral medication In patients with low to intermediate risk MDS who are transfusion dependent

Consider enrolling in this study if you have lower to intermediate risk MDS with an SF3B1 mutation

and require blood transfusions

Participants must meet the following key criteria, among other:

- Diagnosed with low to intermediate risk MDS
- Carries a SF3B1 mutation
- Transfusion dependent
- No prior lenalidomide or hypomethylating agents (HMA) such as azacytidine or decitabine
- Failed or refractory to erythropoiesis stimulating agents (ESA)

If you are interested in participating in this trial, please talk to your doctor about your eligibility and further details about the study

For more information, visit ClinicalTrials.gov and search NCT02841540

RVT-2001 is an investigational drug that is not approved by the FDA or any other global health authority in MDS. Safety and efficacy have not been established in MDS.

**NOW ENROLLING:** A Clinical Study for People with HMA Refractory Higher Risk Mylodysplastic Syndrome (MDS)



#### WHAT IS THE STUDY?

This study is a clinical trial testing an investigational drug called **Eltanexor**. The study's aim is to learn the effects of eltanexor in people with HMA Refractory, Higher Risk Myelodysplastic Syndrome (MDS).

#### WHY IS IT HAPPENING?

The researchers are working to discover whether the investigational drug is effective and safe for patients with Myelodysplastic Syndromes (MDS). In this study, the researchers will be concentrating on HMA Refractory Higher Risk MDS.

### WHO CAN TAKE PART and WHEN DOES IT START?

The study is currently open to adults aged 18 or older with HMA Refractory, Higher Risk Myelodyplastic Syndrome (MDS). Your doctor can fill you in on the other requirements and details of what it is like to participate.

#### WHERE IS IT TAKING PLACE?

This is a global study that will enroll approximately 83 participants at about 56 clinical sites, located in Europe, North America and China.

@2022 Karyopharm Therapeutics Inc. | For more information please visit ClinicalTrials.gov Identifier: NCT02649790 Eltanexor (KPT-8602) is an investigational compound. Efficacy and safety have not been established. There is no guarantee that eltanexor will be approved by the FDA or other global health authorities.









### Have Questions About Our Program or Possible Financial Support?

If you have been prescribed a BMS medication and have questions about your coverage, BMS Access Support® may be able to help. You and your healthcare provider can complete an enrollment form to learn about programs that may be right for you. Visit our website or contact BMS Access Support to learn more.



Call Bristol Myers Squibb Access Support at 1-800-861-0048, 8 AM to 8 PM ET, Monday—Friday



Visit www.BMSAccessSupport.com

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.

Have you recently been diagnosed with higher-risk myelodysplastic syndrome (MDS) and been advised to receive one of the following medications: azacitidine, decitabine, or an oral cedazuridine/decitabine combination?



Now enrolling: the STIMULUS MDS-US clinical trial, a research study evaluating an investigational therapy, sabatolimab, in combination with hypomethylating agents for patients with higher-risk MDS.

#### Participants must meet the following criteria:

- · 18 years of age or older
- · Diagnosed with intermediate-, high-, or very high-risk MDS
- Have not received prior treatment for MDS
- · Have not had a prior organ or stem cell transplant
- Are not eligible for a stem cell transplant or intensive chemotherapy

If you are interested in participating and think you meet these criteria, please contact your doctor to discuss your eligibility for the **STIMULUS MDS-US** clinical trial.

For more information, visit

ClinicalTrials.gov/ct2/show/NCT04878432

or search "STIMULUS MDS-US" in any internet browser or call 1-888-NOW-NOVA or 1-888-669-6682.

Study drug(s) are either investigational or being studied for (a) new use(s). Efficacy (how the drug may help) and safety have not been established, and there is no guarantee that the study drug will become commercially available for the use(s) under investigation.



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### **Advancing Research & Patient Care**



