SEE PAGE 24 FOR PROFESSIONAL SECTION

MDS Foundation

INTRODUCING NEW LOOK!

VOLUME 29 ISSUE 2
FALL/WINTER 2023

NEWSLETTER

The Myelodysplastic Syndromes Foundation, Inc.

THANK YOU 2023!

We met our fundraising goal of $400k, stay tuned for 2024.

Move FOR MDS

Community Walks to Drive Awareness & Accelerate Research

PLAN TO ATTEND

18th INTERNATIONAL CONGRESS ON MYELODYSPLASTIC SYNDROMES

7–10 May 2025, Rotterdam, The Netherlands

In This Issue

PATIENTS & FAMILIES SECTION

PROFESSIONAL SECTION

Meeting Highlights & Announcements

Research

Do you know...

Do You Know Your MDS Subtype, IPSS-M Score & Gene Mutation Profile?

MDS treatment is individualized based on a patient's subtype, IPSS-M score and, to some extent, genetic mutations. 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-M score are determined, as well as general information on genetic mutations commonly found in MDS and the importance of genetic testing for these mutations.

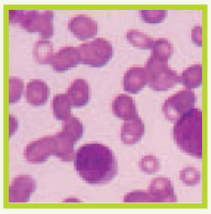
Knowing your subtype, IPSS-M score and gene mutation profile will help facilitate discussions with your healthcare provider on what this means for you personally and help select the best treatment options.

IPSS-M Score

The IPSS-M 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.



Bone Marrow Blast

MDS-RS-MLD

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.

KNOWLEDGE IS POWER

Visit Our Microsite

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 learn more, visit our website at https://www.mdsknowledgeispower.com/.

To order your free copy of UNDERSTANDING YOUR MDS: Know your Score, your Subtype, and your Mutation, please call 1-609-298-1035 or scan the QR code.

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Move FOR MDS

Community Walks to Drive Awareness & Accelerate Research

Ready for 2024?

Stay tuned to find out next year's locations!

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Clinical Trials in MDS

New modules coming soon to YouAndMDS.com

Developed by the Myelodysplastic Syndromes Foundation, Inc. and Mechanisms in Medicine Inc.

MDS Foundation

The Myelodysplastic Syndromes Foundation, Inc.

Questions about MDS? Need support or resources?

Contact our Director of Patient Care now.

You are not on your own. The MDS Foundation supports and educates patients, communities and healthcare providers. We help accelerate innovative research in MDS and its related diseases to better diagnose, control and ultimately cure them. We can help you. We are the people who make hope work.

Educate • Communicate • Advocate

Ashley Moncrief RN, BSN, Director of Patient Care:
1-800-637-0839 ext. 210 • amoncrief@mds-foundation.org

mds-foundation.org

SparkCures

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

Find MDS Clinical Trials Across the US

Using the NEW MDS Clinical Trial Matching Platform

The Clinical Trial Process

Identification | Screening | Enrollment | Treatment | Follow-Up

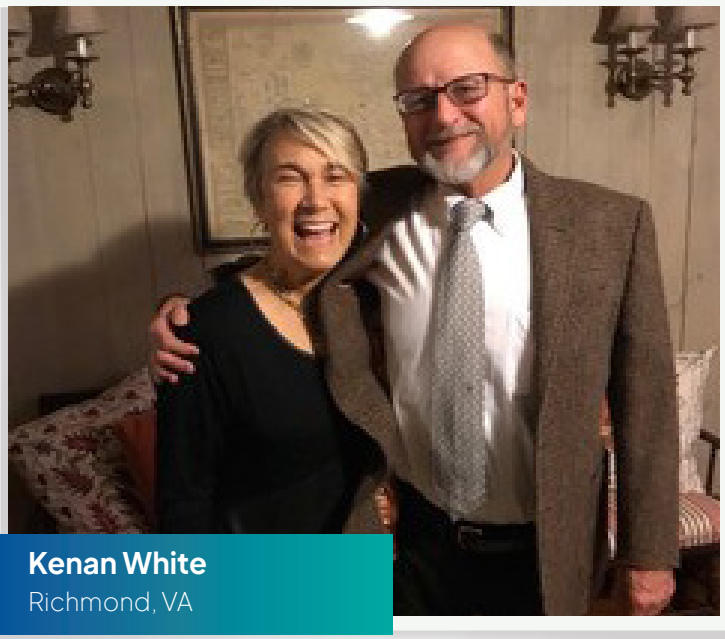
The MDS Foundation is proud to partner with SparkCures, LLC to develop a Clinical Trail Matching Platform. This revolutionary platform will offer fast and personalized clinical trial screening and matching services, simplifying the complex landscape of clinical trial options for MDS patients, caregivers, and healthcare providers.

For more information visit https://mdsf.sparkcures.com/

Patient Story

The Land of MDS

We come to the land of MDS via many routes: bruising, bleeding, fatigue, infections, rashes, shortness of breath, odd results on a routine blood test, an astute doctor tells you "do you know you are yellow?," as well as indisputable evidence via blood tests or a bone marrow biopsy.



Kenan White
Richmond, VA

THE DIAGNOSIS

Indisputable evidence – "Sorry Mrs. White, but your biopsy shows that you have MDS."

No primer on MDS, no 30–minute explanation of a disease so complicated that I can't even pronounce its 'proper name,' no 'I know that this is difficult, but … .'

Everyone's tale is a bit different, but in my case, it began there and whatever came next in the 10 minutes my husband and I had with the doctor sounded like: 'sorry … blah, blah, blah… low risk… blah, blah, blah … yes, it's considered a cancer … blah, blah, blah… unfortunately no cure … blah, blah, blah… you could try a stem cell transplant but … blah, blah, blah….'

THE AFTERMATH

Where would we be without Dr. Google, and I'm being completely facetious. It might be a rare disease but there is plenty of information to send you into the land of anxiety, panic, fear, and hopelessness. We've all been there, but the first question is: 'how long do I have?' and that is not the question to ask. I promise. So, with little to no understanding about my particular situation, I began a deep dive into the rabbit hole and stayed down there long enough to get very depressed.

THE REMEDY (not recommended)

I could have spared myself a great deal of angst if I had known what I know now, but I was out there on my own. My first inclination was to shut down: everyday, after work, I would come home and binge on episode after episode of The Office. This was my way of numbing my brain and escaping whatever fears and anxieties I had allowed into my head. It was not the worst coping strategy, but after 9 seasons, I began to wake up to a new reality. This was going to be my life and I better get going.

A BETTER REMEDY

During this time, I was being monitored by my doctor at the VA Cancer Center, and I was beginning to come out of the fog. My first step was to clear my head, so to speak, and I found a wonderful therapist who deals specifically with grief.

And if MDS was my disease, grief was one of its manifestations. I worked hard to get my head around it, and then even harder to find it a place in my life, a place where it wouldn't get in the way of the life I had left.

NO CONTROL, NO WAY

When my GP (general practitioner) advised me that this was a disease that I had no control over, she had basically thrown down a gauntlet. Yes, I understood that this was an incurable disease and there was nothing I could do to change the inevitable - that was up to the specialists and medications. But if she thought that someone like me, whose middle name is CONTROL, was going to sit back and let this run its course, well she was mistaken. I had already found the healthy tools to deal with the anxiety and depression, and now I was going to work on the other challenges that MDS brings to the table. The easy things: diet, exercise, and sleep. The harder things: fatigue, letting things go and letting people in.

TALK IS CHEAP

My GP is a gem, and her words weren't meant to hurt. She gave me even better advice: talk is cheap. So, I began talking and listening. MDS is a rare disease, but there are so many smart people out there working on it – worldwide. And, finally, the internet became my ally as I began some serious research and discussion and second opinions. I went to Johns Hopkins. I went to Dana Farber and contacted folks 'in the know.' All concluded that my course of treatment was the one that they would recommend, and I felt more in control than ever before. I've also realized that what works for me may not work for all, so I will avoid a discussion of my 'course of action.' I will say this: when one thing stops working, there are more things to try and try we must.

Patient Story

MDS Foundation
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A LONELY DISEASE

MDS is an exclusive disease. 'You have WHAT? Never heard of it.' Yes, most haven't. We are exclusive, but that is not a good thing. It's a complicated disease, it's a rare disease, and it is a lonely disease. Support groups are not in every city, and you won't meet many people in your world that have come into contact with the disease, much less know a fellow traveler. Organizations like the MDS Foundation are invaluable for patients wanting a bit of connection and support and information. Infusion rooms are not filled with MDS sufferers, but they are filled with people facing challenges and their determination and hopeful outlooks and faith and good cheer (not to mention the nurses and doctors who are on your team) remind me that this may be what they mean by 'God closes a door, but he opens a window.'

A BLESSING (WELL, SORT OF)

I won't kid myself or you. If I could shuck this disease, I would in a red-hot second. But, that is not an option so let me tell you a few good things that have happened. I love my life, now more than ever. I've prioritized my relationships, and I don't miss an opportunity to connect with the people I love. I'm not in a hurry and I don't fret when things go wrong (well, that's a goal as we all know, waiting for the results of our latest biopsy or blood test or whatever measurement determines our next step.) I tend to say yes to most things. I take such joy in the smallest things. I'm beginning to relish those seemingly trite quotes you find on plaques and mugs and tee shirts: don't sweat the small stuff, seize the day, everything happens for reason … you know them all. Whoever came up with them must have had some challenges! But, I don't get bothered by small things. I do wake up every morning feeling grateful even though I feel crummy; I am grateful for everything in my life. I do avoid negativity when I can and I do live each day as it was my last. I'm sure that there are a few close to me that may call me out on a few of these, but hey, it's my article!

LAST WORDS

MDS does not define me, therefore I try very hard not to let it into my daily life. There are few signs of the disease, and it's easy for me not to make it front and center with family and friends. I've made that a priority; I know that I'm surrounded by love and support and could call on so many if I needed them. Bless them all.

As hard as I try not to burden with a 'woe is me' attitude, there are a few in my life that have shouldered the 'bad side' of this journey, and I'd be remiss in not thanking my incredibly supportive husband who has been with me since day one. We all have that person, and they all deserve recognition because not even I know what these past 5 years have done to his soul and spirit. I try to imagine if our situations were reversed, and I can't. I can only hope that I would be as generous and supportive and patient. My goal has been NOT to let MDS define me as a person, as a victim; therefore, there are so many aspects of this disease that only he is aware of and I will be forever in his debt for helping me shoulder the challenge of MDS.

> ***I do wake up every morning feeling grateful even though I feel crummy; I am grateful for everything in my life. I do avoid negativity when I can, and I do live each day as it was my last.***
>
> Kenan White

My Experience with the MDS Foundation

Suresh Dutia
Vienna, VA

I experienced a drastic drop in my platelets after the COVID Booster.

Following several blood tests & a bone marrow biopsy, I was diagnosed with low-grade myelodysplastic syndrome (MDS) mainly affecting platelets. I started searching for more information online and stumbled upon the MDS Foundation Website. After reviewing information, I contacted Ms. Ashley Moncrief, Director of Patient Care, and explained my case and all relevant test results. With the help of Ms. Moncrief, I was immediately connected with MDS Center of Excellence at Johns Hopkins University School of Medicine. This experience alleviated my anxiety about MDS and rendered moral upliftment & positive feelings to deal with my health. My overall experience with the MDS Foundation is excellent. Further, I also reviewed information on the MDS Foundation website in various Languages, and it is very impressive.

Hope doesn't work just by wishing. That's important to know for fighting MDS, a challenging but little-known cancer that, left undetected, can progress to acute myeloid leukemia and other forms of blood cancer. MDS is not incurable. It simply hasn't been cured yet. But with your help, that day is coming.

Your donation turns hope into reality. For nearly 30 years, The Myelodysplastic Syndromes Foundation has been a catalyst for progress: Supporting patients. Expanding education. Accelerating research. Bringing critical awareness of MDS to the world. We depend on your investment to make this progress happen. Donate today. And make hope a life-changing force. **Give at MDSdonate.org**

Scan to Donate
MDSdonate.org

We are the people who make hope work.

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

COMING IN 2024...

Make sure to visit our website at WWW.MDS-FOUNDATION.ORG for news on upcoming events

WANT TO HAVE A PATIENT FORUM NEAR YOU?

Reach out to our **Director of Patient Care, Ashley** (Amoncrief@mds-foundation.org), to advocate for a spot in your community!

UPCOMING MDS PATIENT FORUM, WEBINAR & PODCAST TOPICS WILL INCLUDE:

- Understanding the Genetics: Reading NGS Reports, IPSS-M, Targeted Therapies, Cytogenetics (Good vs.Poor Risk)
- Review of the WHO 2022 Classifications
- History of MDS Drug Approvals & Drug Classes for MDS
- Emotional Support: Role of Mental Health Professionals, Starting a Support Group, Caregiver Support
- Homeopathic Therapy
- Transfusion Basics: Purpose, Long Term Impact, Iron Chelation, Irradiated Products, Tracking
- Treatment Option Types: Watch & Wait, Curative (HSCT), Supportive Care, Disease Control, Palliative Care, Hospice
- Oncologic Emergencies in MDS & Infection Basics
- MDS Foundation Basics: Getting the Help you Need
- Professional Conference Patient Summaries & Clinical Trial News
- Being Your Own Advocate

JOE IS GETTING AN UPGRADE!

JOE in MDS, short for '**J**ourney **O**f **E**mpowerment', launched in March 2023. Since then, we have listened to the insights provided by patients, caregivers, and healthcare professionals to improve the platform and enhance the learner experience.

We will be making updates to **JOE in MDS** throughout 2024, aligning the platform with the feedback we received from the MDS community.

- New modules (including nutritional information)
- Improving user experience
- Content updates
- More visuals and diagrams
- Resource section
- Tailored learning
- Updated dashboard
- New quiz questions

VISIT TODAY

mdsJOE.com

SCAN ME

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

JOE

#mdsJOE

MDS JOE is brought to you by the MDS Foundation

PLEASE SCAN TO RECEIVE YOUR GUIDE

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.

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.

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

In partnership with CCO powered by cea

Smarter. Stronger. Together.

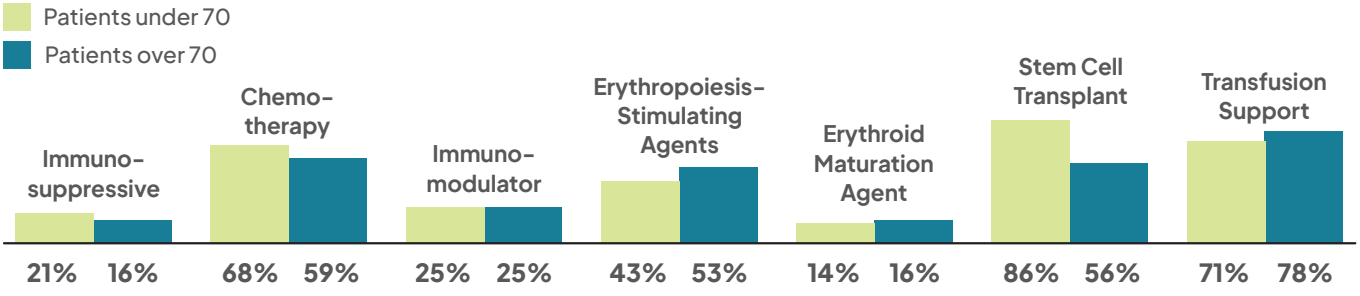
A diagnosis of Myelodysplastic Syndrome (MDS) is a shock to anyone. The MDS Foundation is here to support patients and their families as they navigate their care options. We provide information about the disease, work with healthcare providers, and fund research.

The MDS Foundation strives to learn from patients' experiences. In partnership with Clinical Care Options, LLC., the MDS Foundation surveyed patients with MDS about their perceptions of treatment and care. The following results showcase patients' concerns during the treatment process and what they would like from their care team. Together we are ensuring the patient voice is heard!

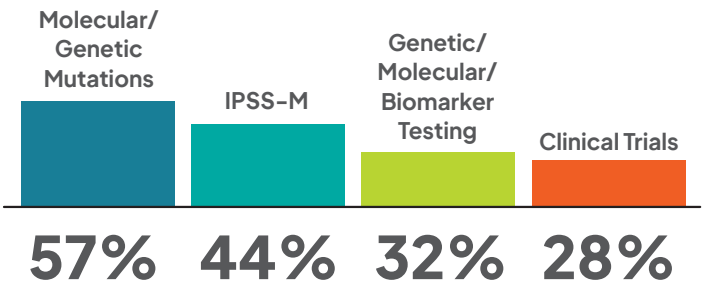
Understanding MDS and Treatment Options

MDS is a complex disease, taking many forms. The treatment options are just as varied and can be confusing. Survey results show that most patients have discussed multiple treatment options and diagnosis tools with their healthcare team.

Therapies Care Team Discussed with Patient



Risk and Prognosis Tools Discussed with Patients



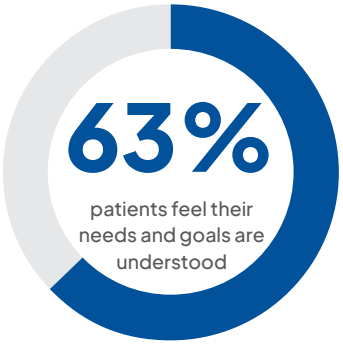
MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

In partnership with CCO powered by cea

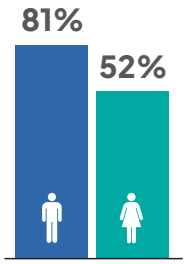
Patients Comfort with Care Team

As a patient, being heard by your healthcare team is essential. Our findings show that most patients feel their needs and concerns are being met by their healthcare providers. Patients feel informed about treatment options and that their values and treatment goals are understood.

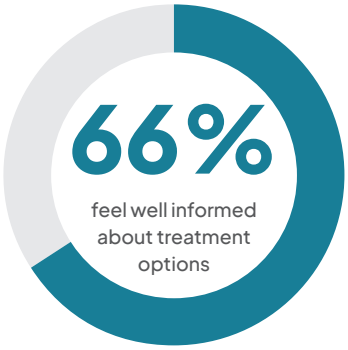
Feeling Understood



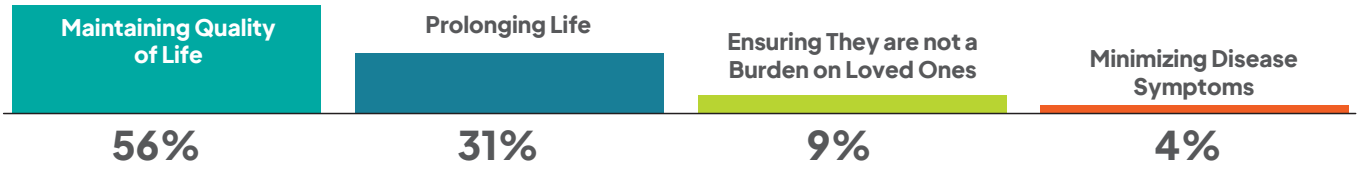
More male patients felt understood than female.



Well Informed and Appropriate Treatment

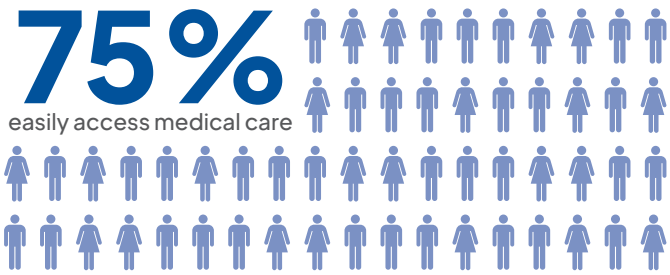


Patient's Most Important Treatment Goal

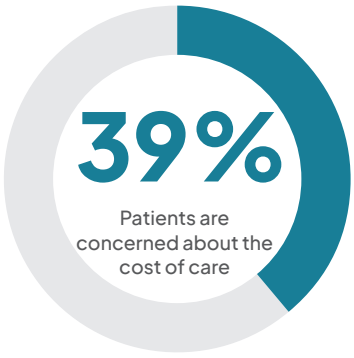


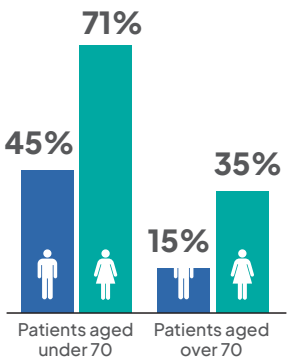
Accessibility and Cost of Care

When you are diagnosed with MDS, the last thing you want to think about is how to access treatment and the cost of care. Fortunately, most of the survey participants have easy access to care, and cost is a concern to less than half.

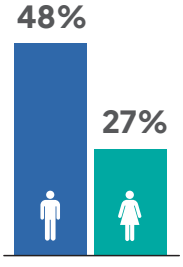


Concerns About Cost





Discussed Insurance or Financial Assistance



This survey was conducted by MDS Foundation in partnership with CCO in the fall of 2023. Survey participants characteristics: Age: 50–59, 10%; 60–69, 25%; 70–79, 43%; 80+, 22%. Gender: men 39%; women 61%. Race: white 95%; other 5%.

Coming Soon

JOE and AML

Keep an eye out for upcoming announcements!

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

MDS JOE is brought to you by the MDS Foundation

Shared **experiences** have the power to **change the story** for this generation of patients, caregivers and survivors, **and the next.**

Voice them.

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!

Use the QR code to take the survey!

CancerSupportCommunity.org/Registry

APR
21

AML

World Awareness Day

AML World Awareness Day is the one day of the year that people from all around the world come together to help raise awareness of acute myeloid leukemia (AML) — a rare, aggressive cancer of the blood and bone marrow and the most common form of acute leukemia in adults.

KNOW AML

The first global education and awareness initiative that provides patients and caregivers with the information, resources, and support they need to deal with AML.

Know AML has a dedicated and highly experienced global ambassador group who meet regularly to steer and guide the initiative. The ambassador group consists of patients and caregivers, patient advocates, healthcare professionals, and industry representatives. Their responsibility is to propose awareness initiatives, provide concepts for support and education resources, share their experiences and learnings from advocacy heritage, and endorse materials before circulation among the AML community.

Scientific Education Support | ALAN

Know AML is brought to you by Scientific Education Support (SES) in collaboration with the Acute Leukemia Advocates Network (ALAN).

know-aml.com

The Results Are In...

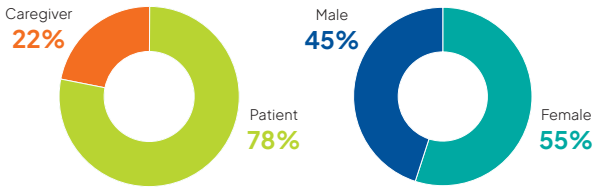
The Myelodysplastic Syndromes Foundation (MDSF) has conducted a patient and caregiver survey from 2015 – 2023. Over the course of this time, a total of 597 participants completed the survey.

Who Took the Survey?

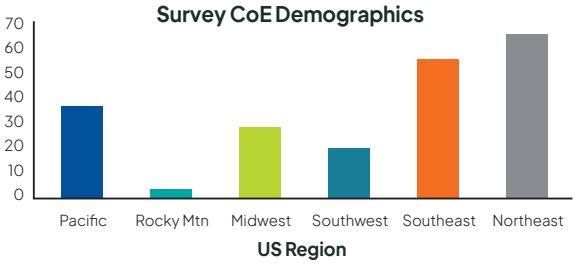
The demographics of the survey were as follows:

Average patient age	Average caregiver age	Range for year of diagnosis
69	61	1985–2022

Participant Type and Gender Identification



An estimated **52.67%** of the respondents reported having been seen at a MDS Center of Excellence. The four centers with the most survey respondents were as follows: Moffitt (17 participants), Fred Hutchinson (17 participants), Columbia (14 participants), and Stanford (11 participants). Although the majority are seen at Centers of Excellence, **71.57%** receive treatment at a local hematology clinic; thus, demonstrating an ongoing partnership between the two. The survey revealed that the foundation is reaching people primarily in the Northeast and Southeast regions of the United States, with the least number of participants located in the Rocky Mountain region.

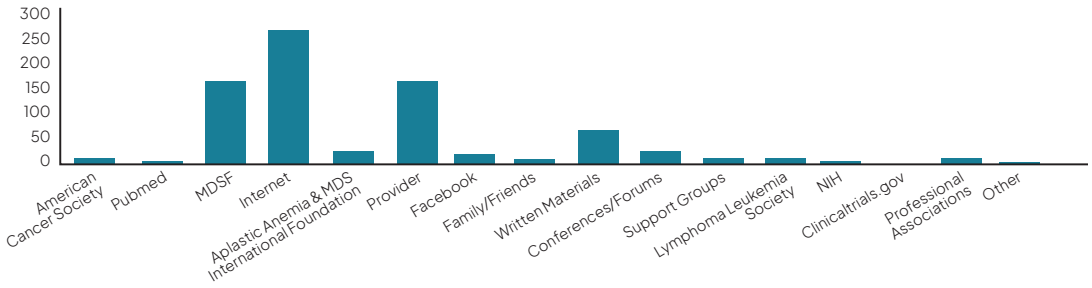


What were the Results?

Participants were asked about clinical trials. An estimated **61.19%** of respondents reported that they have considered participating in a clinical trial, while only **13.88%** have been a trial participant. The majority of patients, **59.21%**, reported that their provider had not discussed clinical trials with them and **60.62%** stated that they receive information on MDS clinical trials from the MDS Foundation. The internet was another common source of trial knowledge; **12.6%** of those surveyed reported finding data from internet searches.

Given the rise of technology utilization in healthcare, questions were added to the survey to determine how the MDS patient and caregiver population has incorporated digital platforms into their care. Between **70 and 80%** of those surveyed reported using a smartphone daily; **30–40%** reported daily use of a tablet. Despite the popularity of handheld devices, **78.41%** still preferred printed health information. As respondents were allowed to select more than one modality for education delivery, it is important to note that **71.36%** reported that they would prefer written material online. The takeaway is that patients are seeking health literature rather than visual or interactive venues. When asked where they go to for health information, the following were the top three contenders: the internet (between 250–300 respondents), the MDS Foundation (between 150–200 respondents), and healthcare professionals (between 150–200 respondents).

Information Sources Used by MDS Patients



The most important questions of the survey resolved around what patients and caregivers have identified as the gaps in care. Of note, only 6.5% of respondents were satisfied with the currently available resources. Per those surveyed, the top three areas of need identified were the following:

COMMUNITY

✓ **Emotional Support (23.2%)**

- Caregiver resources
- Generalized emotional support
- More support groups
- Other patients' stories/ experiences
- Information on impact on younger patients

QUALITY

✓ **Better format & delivery (21.8%)**

- New printed information
- Less medical jargon
- More patient forums and events
- Increase in internet-based resources
- Update of the MDSF website
- Information provided in more languages

AWARENESS

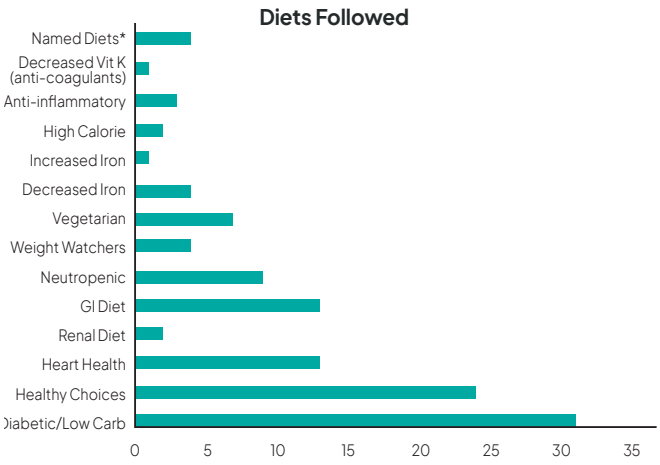
✓ **Advances in MDS (16.7%)**

- International and global advances in treatment
- New updates in the MDS space
- Homeopathic therapies
- Clinical trials
- Access to research articles
- Help identifying reputable sources
- MDSF news

MDS Foundation Patient and Caregiver Survey

As treatment for MDS becomes a priority, it is important to remember that patients have other co-morbidities and healthcare needs which must be met outside of the realm of hematology. It was encouraging to see that **69.90%** of respondents had seen their primary care physician within the past three months. **78.31%** reported seeing other medical specialists on a routine basis. The 368 participants who answered this question identified a total of 758 medical specialists; this equates to ~2.05 sub-specialties per person. The top three medical disciplines noted by MDS patients in the survey were cardiovascular (**35.8%**), urinary/reproductive (**33.9%**), and integumentary (**26.6%**). Polypharmacy is a concern for this medically complex group. When asked how many prescription medications are being taken, the average was 4 per person. When factoring in over-the-counter medications, the 403 patients who answered this question identified the following as the top three categories of non-prescription medications taken: vitamins (**68.2%** of those surveyed), medications for heart health (**24%** of those surveyed), and pain management (**17.6%** of those surveyed).

It became apparent that MDS patients are proactive in their healthcare. **87.66%** reported attending classes aimed at health and wellness. **77.45%** reported following a special diet with the majority adhering to a low carbohydrate/diabetic diet (between 30–35 patients). Despite the focus on nutrition, adequate caloric intake remains a challenge with **51.88%** of patients reporting a poor appetite.



A group of questions was focused on quality of life and symptom burden of the disease. Fatigue is a real problem for MDS patients with **28.57%** waking up tired each morning and **48.79**% feeling tired at the end of the day. Approximately **25%** reported having trouble starting things due to fatigue; an equal number reported difficulty ending projects for the same reason. **59.56%** reported needing medication to assist with insomnia. The end result is that **35.46%** of those surveyed reported limiting activities due to fatigue.

The psychological impact of MDS can be life altering. The majority of patients (**55.46%**) reported feeling isolated and alone. **33.56%** experience anxiety, **37.03%** experience depression, and **35.19%** experience uncertainty; this does not take into account those who are not comfortable with reporting the mental health impact of the disease. 26.06% of those surveyed reported relying on family and friends to complete activities of daily living. Despite the challenges, it is encouraging to hear that **45.43%** of those surveyed remain hopeful. Those impacted by MDS prove to be consistently resilient.

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

Where do we go from Here?

The MDS Foundation is in the process of using these survey results to develop a targeted outreach approach. The focus is on emotional support. Support group information, to include a leadership toolkit, is now being promoted at patient forum events. The Director of Patient Care is making an effort to clean up support group information, join support group calls, and facilitate the development of new groups. In addition, the MDS Foundation is updating and promoting Colloquy, a social media platform created for and dedicated to those impacted by MDS.

Improving information delivery is also a top priority. Stay tuned for reconstruction updates for the foundation website. The MDS Foundation has partnered with IPNY to improve marketing and communication efforts. MDS Foundation supported publications including the Building Blocks of Hope and Guide to Assistance Programs are being updated and will be ready for distribution soon; part of this update will involve translations into other languages to include Japanese.

Given the interest in clinical trials, the foundation is working to distribute accurate, easy to navigate information on MDS clinical trials to patients and providers. The MDS Foundation has partnered with SparkCures to develop a unique platform which allows patients and providers to search for clinical trials based on individual disease characteristics and location. Patients and providers can create accounts, set up phone appointments with SparkCures and MDSF staff, and filter by commonly used trial criteria. Filters include center, subtype, treatment history, risk level, drug classification, trial phase, trial type, organ function, and more. As new drugs are approved, the foundation staff are dedicated to the distribution of press releases. In addition, the MDS Foundation has close partnerships with pharmaceutical companies and plans to continue serving as advisors on the patient experience. One such example of this was the partnership of Bristol Myers Squibb and the MDS Foundation for a presentation at the Rebloyzl Relaunch in September 2023 after the drug was approved as a front-line therapy.

Partnerships are important to nonprofit organizations. The MDS Foundation plans to strengthen its relationship with the Centers of Excellence by creating a culture of community. A referral network is being developed and a meeting structure is being established. A one-page flyer for distribution at the centers was created this year as an introduction to the foundation for new patients. The MDS Foundation will also be hosting its first meet-and-greet event for Centers of Excellence representatives at the American Society of Hematology meeting in 2024. As communication among the centers increases, so will patient satisfaction.

In an effort to remain patient focused, the MDS Foundation is now working to update the survey with plans to review the data on a yearly basis. The survey will be re-opened in the very near future. The foundation also plans to release quarterly patient and caregiver interaction metrics which will give details on the number of patients impacted.

PleXus Communications is pleased to partner with the **MDS Foundation** on the **Community Hematology/Oncology Forums**

This activity is supported by an educational grant from Bristol Myers Squibb.

Optimizing Therapy for Low-risk Myelodysplastic Syndromes in Community Practice

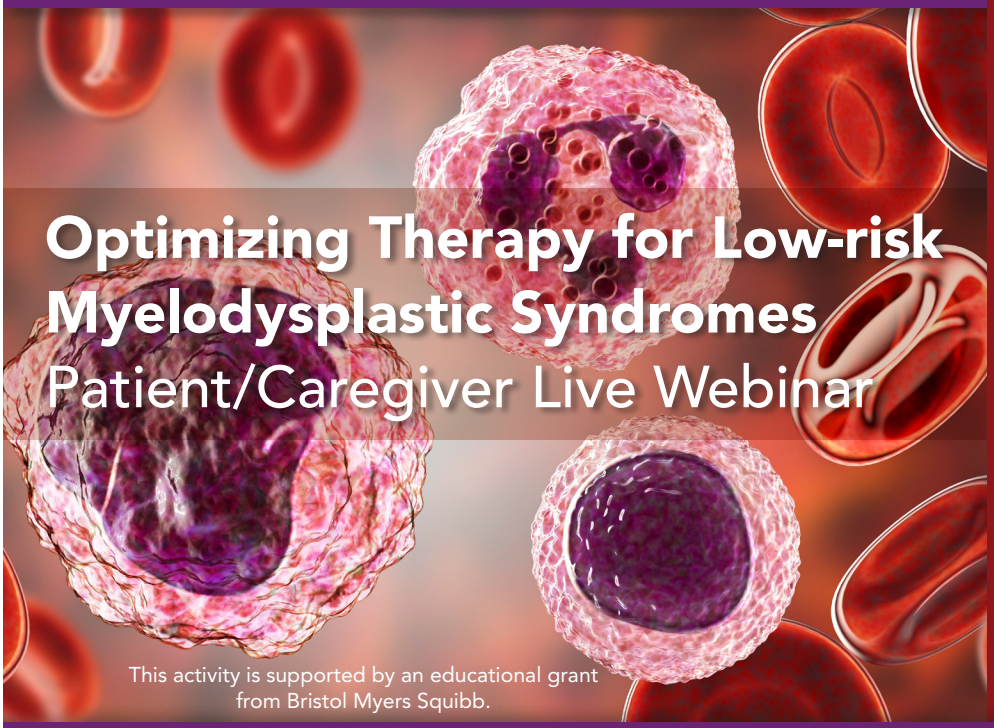
Request a free live (in-person or webinar) expert-led **CME/CE-accredited presentation** for your hem/onc department.

Email **info@plexuscomm.com** for additional info and/or to request a program

MDS Foundation The Myelodysplastic Syndromes Foundation, Inc. PleXus Communications

Coming in early 2024

This webinar is held in partnership with the MDS Foundation



Optimizing Therapy for Low-risk Myelodysplastic Syndromes

Patient/Caregiver Live Webinar

This activity is supported by an educational grant from Bristol Myers Squibb.

Presenter

Casey O'Connell, MD

Associate Professor of Clinical Medicine, Lawrence and Janice Kelly Chair in Hematology

Medical Director for Anticoagulation Services for LAC+USC

Director, Gehr Cures: Myeloid Malignancy Program of USC

Keck School of Medicine of USC

Email: **mdswebinar@plexuscomm.com** to receive webinar link

MDS Foundation The Myelodysplastic Syndromes Foundation, Inc. PleXus Communications

Notable

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

Learn More About Our Industry Partners

Notable: Understanding and Seeking Answers

Despite comprehensive research efforts around the globe to understand the underlying causes and therapeutic targets of Myelodysplastic Syndromes (MDS), there are currently no cures outside of transplant and no real confirmatory etiology (cause). Myelodysplastic Syndromes refer to a group of disorders in which the bone marrow produces too few mature and/or functioning red blood cells, white blood cells or platelets. It begins with a change to a normal stem cell in the bone marrow. The overall incidence of MDS in the United States is estimated at close to four cases per 100,000 people, with as many as 20,000 to 30,000 people diagnosed annually. There may be 60,000 to 170,000 people suffering with MDS in the US alone.

A central part of understanding MDS is really understanding and seeking answers for patients and through this work, creating the scientific insights for finding the medicine that delivers the greatest and most durable medical impact for the patient. Patients have shared tough parts of their journeys:

"...the years rolled by the appointments got closer together as my illness progressed. I've had sepsis around 14 times. I've had serious line infections from having a Hickman line inserted into my chest which would sit just above my heart. Last year I had 2 types of flu and have had flu every year since I had my transplant as well as a few times before. I've had NG and NJ tubes and still have a PEG J inserted in my tummy due to long periods of not being able to eat." [K.B., MDS patient]

"Another biopsy confirmed Taylor's marrow was still empty and along with the tri-lineal cytopenia confirmed the situation was still dire and maybe progressing. This biopsy also revealed some cytogenic anomalies for the first time (del 7q). It was stem cell transplant time for Taylor. The diagnosis for Taylor was ultimately hypoplastic MDS." [T.B., MDS Patient]

"I had to wait for the disease to decide its course before we could look at treatment options. We found out that the medication that would usually be used, would not work for me... I was nervous but I had seen three different Hematologists / Oncologists, so I knew I had to keep the faith. I kept focusing on one sequence, del (5q), as my white counts and blasts continued to rise." [S.N., MDS Patient]

We at Notable see many common concerns among patients and while each patient's journey is different, many similar obstacles and challenges have compelled us to seek improved, faster answers, especially to one of the most important questions of patients: "Doctor, will this therapy you're recommending actually work for me?"

We believe: "**EACH PATIENT IS NOTABLE AND CAN BENEFIT FROM A PREDICTIVE PRECISION MEDICINE THAT WORKS FOR THEM**". Following our recent merger and public launch on the NASDAQ, we are focusing the resources of the integrated company on advancing and accelerating medical progress for patients with MDS, AML, and other hematological diseases.

Notable's PPMP, our proprietary Predictive Precision Medicines Platform, aims to predict, before treatment, whether a patient will clinically respond to their treatment (or not), is at the heart of what patients want to know and what their clinicians want to determine. We're focused on bringing the clinical community improved, predictable outcomes – while enabling the acceleration of medical decisions and simplifying medical practice.

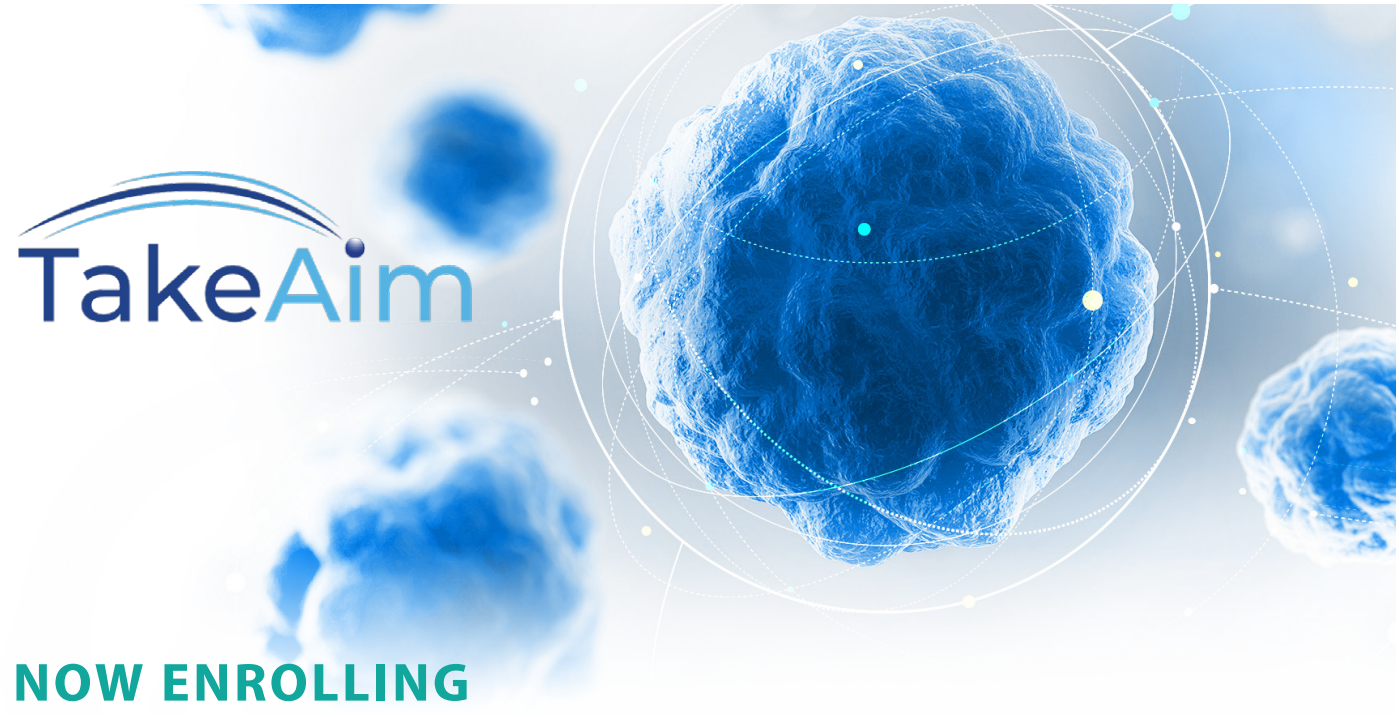
Predicting a patient's clinical response can match treatments with the patients who are most likely to respond, potentially ensuring medical benefit for them and protecting the other patients, who traditionally would have been treated but not responded, from losing time, experiencing toxicity, and achieving better outcomes.

Beyond improving patient outcomes, predicting treatment response advances the development of novel medicines. Selectively enrolling predicted responders into clinical trials should make trials more likely to succeed and should result in trials that are smaller, faster, and less costly. Bringing medicines to patients faster and more predictably benefits all concerned.

Enabled by the combined resources of the two companies, we're expanding our recent strong results of four clinical validation studies in partnership with Stanford University, MD Anderson Cancer Center, Washington University, and Texas Children's' Hospital.

Our team at Notable has built a clinical-stage platform therapeutics company that is developing predictive precision medicines for patients with cancer. With the PPMP, our platform bio-simulates a cancer treatment and predicts whether or not a patient is likely to respond to that specific therapeutic choice. We employ the industry's largest cancer response database without a reliance on genomics and apply machine learning and AI. Our PPMP enables the identification and selection of clinically responsive patients prior to their treatment and thereby potentially enables fast-track clinical development in this patient population. By continually advancing and expanding the reach of the PPMP across diseases and predicted medical outcomes, Notable aims to be the leader in precision medicine and revolutionize the way in which patients seek and receive treatments that work best for them – patient by patient and cancer by cancer.

Notable is proud to be a National Gold Sponsor of "MOVE for MDS 2023". We are headquartered in Foster City, California. Learn more at www.notablelabs.com and follow us @ notablelabs.



TakeAim

NOW ENROLLING
LEUKEMIA TRIAL

TakeAim Leukemia: A Phase 1/2A, Open Label Dose Escalation and Expansion Study of Orally Administered CA-4948 as a Monotherapy in Patients With Acute Myelogenous Leukemia or Myelodysplastic Syndrome

BRIEF SUMMARY:

This is a multicenter, open-label, Phase 1/2a dose escalation and expansion study of orally administered emavusertib (CA-4948) monotherapy in adult patients with Acute Myelogenous Leukemia (AML) or high risk Myelodysplastic Syndrome (MDS).

- FLT3m positive and who have received ≤2 lines of prior treatment
- Spliceosome mutation of SF3B1 or U2AF1 and ≤2 lines of prior treatment

PHASE 1/2A STUDY DESIGN:

PHASE 1A
COMPLETED
1
28-DAY CYCLE

→

PHASE 2A
Recommended Phase 2 Dose
300 mg BID
OPEN
2
28-DAY CYCLE

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

WE ARE | **working relentlessly to develop innovative and differentiated therapeutics that improve the lives of cancer patients**

CURIS®

STUDY OVERVIEW

BRIEF SUMMARY OF PHASE 2A

The Phase 2a Dose Expansion will be in 3 Cohorts of patients:

1. R/R AML with FLT3 mutations who have been previously treated with a FLT3 inhibitor;
2. R/R AML with spliceosome mutations of SF3B1 or U2AF1; and
3. R/R hrMDS (IPSS-R score > 3.5) with spliceosome mutations of SF3B1 or U2AF1.

All patients above will have had ≤ 2 lines of prior systemic anticancer treatment.

TREATMENT CYCLE

Emavusertib tablets are administered twice daily by mouth. Each treatment cycle is 28 days in length and repeated in the absence of toxicity. Patients who tolerate emavusertib may continue to receive emavusertib until progression of disease, intolerable toxicity, lack of clinical benefit, withdrawal from the trial, or study termination.

KEY ELIGIBILITY CRITERIA

(Please speak with your healthcare provider for the full list of inclusion & exclusion criteria)

INCLUSION CRITERIA:

- Males and females ≥18 years of age
- Life expectancy of at least 3 months
- Eastern Cooperative Oncology Group (ECOG) Performance Status of ≤1
- Cytomorphology based confirmed diagnosis of MDS or AML (as per WHO 2016 classification) with the following characteristics.

  Phase 2a Dose Expansion (Monotherapy)

  Patients with:
  - R/R AML with FLT3 mutations who have been previously treated with a FLT3 inhibitor
  - R/R AML with spliceosome mutations of SF3B1 or U2AF1
  - R/R hrMDS (IPSS-R score > 3.5) with spliceosome mutations of SF3B1 or U2AF1
  - Number of pretreatments: 1 or 2
- Acceptable organ function at screening
- Negative serum pregnancy test in women of childbearing potential
- Women of childbearing potential and men who partner with a woman of childbearing potential must agree to use highly effective contraceptive methods for the duration of the study and for 90 days after the last dose of emavusertib
- Able to undergo serial bone marrow sampling and peripheral blood sampling

EXCLUSION CRITERIA:

- Diagnosed with acute promyelocytic leukemia (APL, M3)
- Has known active central nervous system (CNS) leukemia
- Allogeneic hematopoietic stem cell transplant (Allo-HSCT) within 60 days of the first dose of emavusertib, or clinically significant graft-versus-host disease (GVHD) requiring ongoing up titration of immunosuppressive medications prior to start of emavusertib
- Chronic myeloid leukemia (CML)
- Any prior systemic anti-cancer treatment such as chemotherapy, immunomodulatory drug therapy, etc., received within 3 weeks (or 5 half-lives) prior to start of emavusertib. Localized radiation or surgical resection of skin cancers allowed.
- Use of any investigational agent within 3 weeks or 5 half-lives, whichever is shorter, prior to start of emavusertib
- Presence of an acute or chronic toxicity resulting from prior anti-cancer therapy, with the exception of alopecia that has not resolved to Grade ≤ 1within 7 days prior to start of emavusertib; presence of any acute or chronic non-hematological toxicity ≥ Grade 3 at Screening, or prior to start of emavusertib must resolve to ≤ Grade 2.
- Pregnant or lactating

POSSIBLE RISKS

All study drugs have the potential to cause side effects. Before deciding to enroll in this study, all patients should carefully consider the potential benefits and risks with the healthcare provider. Study doctors will review the risks with every patient and will closely monitor all enrolled participants for side effects throughout the study.

WELCOME TO THE NEW **PROFESSIONAL SECTION** OF THE MDS FOUNDATION NEWSLETTER

Meeting Highlights and Announcements

POST CONGRESS REPORT:

The 17th International Congress on Myelodysplastic Syndromes

MARSEILLE, FRANCE • 3-6 MAY 2023

PARTICIPANT STATISTICS

516 delegates from **39** countries spanning the globe participated.

Top 10 Countries Attending (n):

- United States **(108)**
- France **(10)**
- Germany **(39)**
- United Kingdom **(34)**
- Spain **(33)**
- Canada **(23)**
- Italy **(18)**
- Greece **(14)**
- Sweden **(1)**
- Czech Republic **(13)**

Participants by Region (n):

- Western Europe **(298)**
- North America **(131)**
- East Asia & Pacific **(34)**
- Eastern Europe **(22)**
- Central & South America **(16)**
- Middle East **(13)**
- Central Asia **(1)**
- Africa & Atlantic **(1)**

Participants by Age (%):

- 18–30 years old **(10%)**
- 30–40 years old **(22%)**
- 40–50 years old **(32%)**
- 50–60 years old **(17%)**
- 60+ years old **(19%)**

PARTICIPANT STATISTICS (cont'd)

Participants by Professional Role (%):

Clinical Practitioner **(33%)**

Clinician Researcher **(24%)**

Basic Science Researcher **(12%)**

Other **(8%)**

Student **(8%)**

Industry/Corporate Professional **(7%)**

Resident/Research Fellow **(4%)**

Nurse/Healthcare Practitioner **(4%)**

Participants by Professional Interest (%):

Hematology **(73%)**

Other **(8%)**

Oncology **(7%)**

Medical Genetics and Genomics **(5%)**

Biochemistry/Molecular Biology **(4%)**

Pathology **(2%)**

Pediatrics **(1%)**

Clinical Pharmacology **(0%)**

Participants by Workplace (%):

Hospital **(51%)**

Laboratory **(12%)**

University Hospital **(10%)**

Industry **(9%)**

Research Institute **(6%)**

Comprehensive Care Clinic **(4%)**

University **(4%)**

Other **(4%)**

Government Agency **(0%)**

Private Practice **(0%)**

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

SCIENTIFIC PROGRAM

Number of Sessions per Session type:

13	6	3	2	1
Plenary Sessions	Meet the Expert Sessions	Work-shops	Nurse Program Sessions	Young Investigators Award Session

In total the congress had 67 Invited Speakers and Moderators from 10 countries, with some appearing in several sessions.

Canada • France • Germany • Israel • Italy • Japan • Netherlands • Spain • Sweden • USA

ABSTRACT SUBMISSION

194 Total Abstracts Received

United States • Spain • Brazil • Germany • Italy
France • Greece • Canada • United Kingdom • China
Czech Republic • Japan • Sweden • Argentina
Israel • Australia • South Korea • Taiwan • Urugua
Austria • Uzbekistan • Algeria • Armenia • Chile • India
Jordan • Mexico • Qatar • Romania • Singapore

INDUSTRY SYMPOSIA

COMPANY	SYMPOSIA TYPE	ATTENDANCE
ABBVIE	Plenary	130
ASTEX	Pipeline	85
BMS	Plenary	110
KEROS	Pipeline	85

SPONSORSHIP & EXHIBITION

9 sponsors

Diamond	Bristol Myers Squibb
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KYOTO 2024

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Advancing Research & Patient Care

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This is a Friday Satellite Symposium preceding
The 65th American Society of Hematology
Annual Meeting

Breakfast Symposium

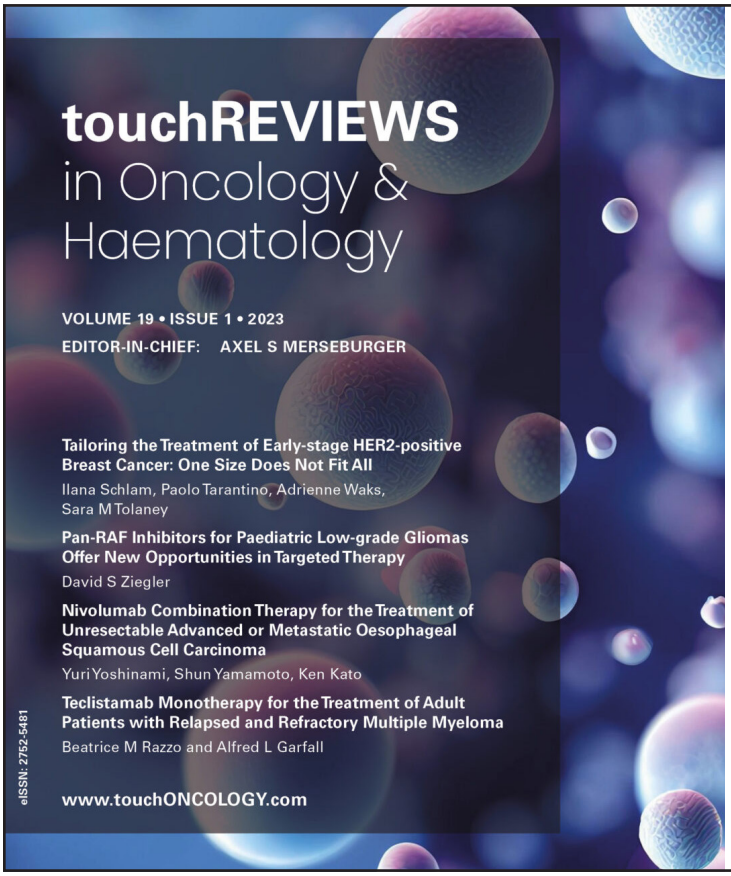
Myelodysplastic Syndromes 2023: What's New

Friday, December 8, 2023 • 7:00–10:00am

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

AKH Inc.
Advancing Knowledge in Healthcare

This activity is jointly-provided by The Myelodysplastic Syndromes Foundation, Inc. and AKH Inc., Advancing Knowledge in Healthcare.
This activity is supported by an educational grant from Astex, BMS, Geron, Notable, and Taiho.



touch HAEMATOLOGY

Welcome to the latest edition of *touchREVIEWS in Oncology & Haematology*! This issue features some of the recent key developments in oncological and haematological disease, including expert interviews, editorials and review articles exploring the latest in lung cancer, pediatric oncology, breast cancer, colorectal cancer, esophageal squamous cell carcinoma, biliary tract cancer, urothelial carcinoma, squamous cell anal carcinoma, cold agglutinin disease and multiple myeloma.

We would like to take this opportunity to thank all who contributed towards this edition, in particular our authors, Editor-in-Chief, editorial board members and partners.

We are now welcoming submissions to our 2023/2024 editions. If you're interested in submitting an article, please get in touch!

Enjoy and happy reading!

https://touchoncology.com/journals/oncology-hematology-review-us/touchreviews-in-oncology-haematology-volume-19-issue-1-2023/

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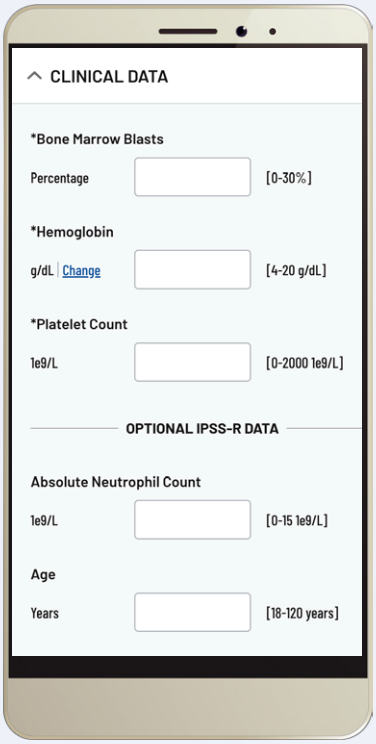
MDS Risk Assessment Calculators

The IWG-PM under the aegis of the MDS Foundation, Inc. has developed two prognostic tools, the IPSS-M and IPSS-R Calculators, to determine a patient's risk of progressing to Acute Myeloid Leukemia (AML).



IPSS-M CALCULATOR

The IPSS-M is the newest MDS prognosis calculator that combines genomic profiling with hematologic and cytogenetic parameters, improving the risk stratification of patients with MDS.

This is a valuable tool for clinical decision-making, offering the prospect of tailoring diagnosis and therapeutic interventions to each patient's molecular profile.

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

DOWNLOAD IPSS-M CALCULATOR APP

https://apps.apple.com/gb/app/ipss-m-risk-calculator/id6447183381

IPSS-R CALCULATOR

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

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

DOWNLOAD IPSS-R CALCULATOR APP

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

MDS Centers of Excellence

Our MDS Centers of Excellence are institutions that meet the highest standards for diagnosis, treatment and patient care. These centers help patients seeking first or second opinions and/or additional treatment options from experts in MDS. We currently have 77 Centers in the United States and 121 Centers in countries around the world.

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

BENEFITS OF MEMBERSHIP:

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

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

TO BE RECOGNIZED AS A CENTER OF EXCELLENCE, AN INSTITUTION MUST HAVE THE FOLLOWING:

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

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

UNITED STATES

ALABAMA

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

ARIZONA

Mayo Clinic Hospital
Phoenix, Arizona
Cecilia Arana Yi, MD/James Slack, MD

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

CALIFORNIA

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

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

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

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

UCLA Health Hematologic Malignancies and Stem Cell Transplant Program
Los Angeles, California
Gary J. Schiller, MD

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

COLORADO

University of Colorado School of Medicine
University of Colorado Cancer Center
Aurora, Colorado
Daniel Aaron Pollyea, MD, MS
Maria Amaya, MD, PhD –
Practice Location:
Rocky Mountain Regional VA
Christine McMahon, MD –
Practice Location: UCHealth Blood Disorders and Cell Therapies Center – Anschutz Medical Campus

CONNECTICUT

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

FLORIDA

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

Mayo Clinic
Jacksonville, Florida
James M. Foran, MD

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

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

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

MDS Foundation Centers of Excellence

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

GEORGIA

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

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

ILLINOIS

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

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

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

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

INDIANA

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

IOWA

The University of Iowa Hospitals and Clinics, Holden Cancer Center
Iowa City, Iowa
Grerk Sutamtewagul, MD

KANSAS

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

MARYLAND

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

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

MASSACHUSETTS

Dana-Farber/Boston Children's Cancer and Blood Disorders Center
Boston, Massachusetts
Akiko Shimamura, MD, PhD

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

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

Tufts Medical Center
Boston, Massachusetts
Andreas Klein, MD

MICHIGAN

Barbara Ann Karmanos Cancer Institute
Wayne State University
Detroit, Michigan
Jay Yang, MD

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

MINNESOTA

Mayo Clinic
Rochester, Minnesota
Aref Al-Kali, MD
Mark R. Litzow, MD
Mrinal S. Patnaik, MBBS

University of Minnesota Medical Center, Fairview University of Minnesota Medical School
Minneapolis, Minnesota
Mark B. Juckett, MD

MISSOURI

Washington University School of Medicine Siteman Cancer Center
St. Louis, Missouri
Matt Walter, MD
Meagan Jacoby, MD

NEBRASKA

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

NEW HAMPSHIRE

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

NEW JERSEY

John Theurer Cancer Center at Hackensack University Medical Center
Hackensack, New Jersey
James McCloskey, MD

Rutgers Cancer Institute of New Jersey
Rutgers University Hematologic Malignancies and Stem Cell Transplant
New Brunswick, New Jersey
Dale G. Schaar, MD, PhD

NEW MEXICO

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

NEW YORK

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

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

Memorial Sloan-Kettering Cancer Center
New York, New York
Aaron D. Goldberg, MD, PhD

Donald and Barbara Zucker School of Medicine at Hofstra/Northwell, Monter Cancer Center
Lake Success, New York
Steven L. Allen, MD

Laura & Isaac Perlmutter Cancer Center at NYU Langone Health
New York, New York
Maher Abdul Hay, MD

Icahn School of Medicine at Mount Sinai
New York, New York
Lewis R. Silverman, MD

New York Medical College/ Westchester Medical Center, Zalmen A. Arlin Cancer Center
Valhalla, New York
Karen Seiter, MD

Roswell Park Cancer Center
Buffalo, New York
Elizabeth Griffiths, MD
James E. Thompson, MD

University of Rochester Medical Center
Rochester, New York
Jane L. Liesveld, MD

Weill Medical College of Cornell University New York Presbyterian Hospital
New York, New York
Gail J. Roboz, MD

NORTH CAROLINA

Atrium Health Levine Cancer Center
Charlotte, North Carolina
Srinivasa R. Sanikommu, MD, FACP
Michael Grunwald, MD

Duke University Medical Center
Durham, North Carolina
Carlos M. deCastro, MD

UNC Lineberger Comprehensive Cancer Center
Chapel Hill, North Carolina
Brandi Reeves, MD

Novant Health Cancer Institute
Charlotte, North Carolina
Patricia Kropf, MD

Wake Forest University School of Medicine Comprehensive Cancer Center
Winston-Salem, North Carolina
Bayard L. Powell, MD

OHIO

Cleveland Clinic Foundation, Taussig Cancer Center
Cleveland, Ohio
Jaroslaw Maciejewski, MD, PhD

Hoxworth Blood Center, George L. Strike Bone Marrow Transplant Program University of Cincinnati – UC Health
Cincinnati, Ohio
Emily Curran, MD

The Ohio State Comprehensive Cancer Center, James Cancer Hospital and Solove Research Institute
Columbus, Ohio
James S. Blachly, MD
Uma M. Borate, MD

PENNSYLVANIA

Allegheny Health Network Cancer Institute Western Pennsylvania Hospital
Pittsburgh, Pennsylvania
Salman Fazal, MD

Fox Chase-Temple University Hospital Bone Marrow Transplant Program
Philadelphia, Pennsylvania
Henry Fung, MD, FACP, FRCPE
Rashmi Khanal, MD, Michael Styler, MD
Asya Varshavsky-Yanovsky, MD, PhD

UPMC Cancer Center
Pittsburgh, Pennsylvania
Anastasios Raptis, MD
James M. Rossetti, DO

University of Pennsylvania Cancer Center
Philadelphia, Pennsylvania
Keith W. Pratz, MD

Sidney Kimmel Cancer Center at Thomas Jefferson University Hospital
Philadelphia, Pennsylvania
Lindsay Wilde, MD

TENNESSEE

Vanderbilt University Medical Center
Nashville, Tennessee
Sanjay Mohan, MD
Michael R. Savona, MD

TEXAS

Texas Oncology – San Antonio
San Antonio, Texas
Roger M. Lyons, MD, FACP
John S. Renshaw, MD

Texas Oncology – Austin Midtown
Austin, Texas
Jason M. Melear, MD

University of Texas, MD Anderson Cancer Center
Houston, Texas
Guillermo Garcia-Manero, MD
Hagop Kantarjian, MD

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

UTAH

University of Utah Huntsman Cancer Institute
Salt Lake City, Utah
Afaf Osman, MD
Paul J. Shami, MD

VIRGINIA

University of Virginia
Charlottesville, Virginia
Michael Keng, MD

WASHINGTON

Fred Hutchinson Cancer Research Center University of Washington Seattle Cancer Care Alliance
Seattle, Washington
Joachim Deeg, MD/Bart L. Scott, MD

WASHINGTON, DC

Georgetown University Hospital Lombardi Comprehensive Cancer Center
Washington, D.C.
Catherine Broome, MD

George Washington University, VA Medical Center
Washington, D.C.
Anita Aggarwal, DO

WISCONSIN

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

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

INTERNATIONAL

ARGENTINA

Sanatorio Sagrado del Corazón
Buenos Aires, Argentina
Marcelo Iastrebner, MD

ARMENIA

Blood Disorders Center of Armenia, Hematology Center after R. Yeolyan
Yerevan, Armenia
Anna Sevoyan, MD

AUSTRALIA

Cabrini Haematology & Oncology Centre
Melbourne, Australia
Melita Kenealy, MD/Gary Richardson, MD

Monash Health Monash University
Clayton, Victoria, Australia
Jake Shortt, BMedSc, MBChB FRACP FRCPA PhD

Peter MacCallum Cancer Institute University of Melbourne
East Melbourne, Australia
John F. Seymour, MD

Royal Hobart Hospital
Tasmania, Australia
Rosemary Harrup, MD

The Royal Melbourne Hospital
Parkville, Victoria, Australia
David Ritchie, MD

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MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

AUSTRIA

Hanusch Hospital
Medical University of Vienna
Vienna, Austria
Michael Pfeilstöcker, MD

Medical University of Vienna
Vienna, Austria
Peter Valent, MD

University Hospital of Innsbruck
Innsbruck, Austria
Reinhard Stauder, MD

BELGIUM

AZ Sint-Jan AV
Brugge, Belgium
Dominik Selleslag, MD

University Hospital Leuven
Leuven, Belgium
Marielle Beckers, MD, PhD
Michel Delforge, MD, PhD

BRAZIL

AC Camargo Hospital–Cancer Center
São Paulo, Brazil
Luiz Fernando Lopes, MD, PhD

Hospital das clínicas da Faculdade de Medicina da Universidade de São Paulo
São Paulo, Brazil
Elvira D. Rodrigues Pereira Velloso, MD, PhD

Universidade Federal de Ceará
Ceará, Brazil
Silvia Maria M. Magalhães, MD, PhD

Universidade Federal de São Paulo
São Paulo, Brazil
Maria de Lourdes Chauffaille, MD, PhD

Hospital Israelita Albert Einstein
São Paulo, Brazil
Nelson Hamerschlak, MD, PhD
Elvira D. Rodrigues Pereira Velloso, MD, PhD

CANADA

Princess Margaret Hospital
Toronto, Ontario, Canada
Karen Yee, MD

Odette Cancer Centre
Sunnybrook Health Sciences Center
Toronto, Ontario, Canada
Rena Buckstein, MD, FRCP
Richard A. Wells, MD

St. Paul's Hospital
Vancouver, British Columbia, Canada
Heather Leitch, MD, PhD

University of Toronto Hospital for Sick Children
Toronto, Ontario, Canada
Yigal Dror, MD

CHINA

Guangdong General Hospital & Guangdong Academy of Medical Sciences
Guangzhou, China
Xin Du, MD, PhD

Institute of Hematology and Blood Diseases Hospital
Chinese Academy of Medical Sciences
Tianjin, China
Zhijian Xiao, MD

The First Affiliated Hospital of Soochow University, Jiangsu Institute of Hematology
Jiangsu Province, China
Suning Chen, MD, PhD

The First Affiliated Hospital of Zhejiang University
Zhejiang Province, China
Hongyan Tong, MD, PhD

The Sixth Hospital Affliated to Shanghai Jiaotong University
Shanghai, China
Chunkang Chang, MD, PhD

CROATIA

Clinical Hospital Merkur
Zagreb, Croatia
Inga Mandac Ragulj, MD

CZECH REPUBLIC

Institute of Hematology & Blood Transfusion
Prague, Czech Republic
Jaroslav Cermák, MD, PhD

DENMARK

Odense University Hospital
The University of Southern Denmark
Odense, Denmark
Klas Raaschou-Jensen, MD
Claus Marcher, MD

Rigshospitalet National University Hospital
Copenhagen, Denmark
Kirsten Grønbaek, MD
Lars Kjeldsen, MD, PhD

FRANCE

Centre Henri Becquerel
Rouen University School of Medicine
Rouen, France
Aspasia Stamatoullas, MD

Centre Hospitalier Universitaire (CHU) de Angers Service des Maladies du Sang
Angers, France
Norbert Ifrah, MD

Centre Hospitalier Universitaire (CHU) de Grenoble
Grenoble, France
Jean-Yves Cahn, MD

Centre Hospitalier Universitaire (CHU) de Limoges Hôpital Dupuytren
Limoges, France
Pascal Turlure, MD

Centre Hospitalier Universitaire (CHU) de Nancy
Nancy, France
Agnés Guerci-Bresler, MD, PhD

Centre Hospitalier Universitaire (CHU) de Tours – Bretonneau
Tours, France
Emmanuel Gyan, MD, PhD

Hôpital Cochin/University Paris V
Paris, France
Francois Dreyfus, MD

Hôpital Saint Louis/University Paris VII
Paris, France
Pierre Fenaux, MD, PhD
Christine Chomienne, MD, PhD

Hôpital Saint-Vincent de Paul (Lille)
Lille, France
Pascal Laurent, MD

Institut Paoli-Calmettes
Marseille, France
Norbert Vey, MD

Service des Maladies du Sang Hôpital Claude Huriez
Lille, France
Bruno Quesnel, MD

GERMANY

Georg-August-Universität Göttingen
Göttingen, Germany
Detlef Haase, MD, PhD

Hannover Medical School
Medizinische Hochschule Hannover
Hannover, Germany
Matthias Eder, MD, PhD

Heinrich-Heine Universität Düsseldorf
University Hospital
Düsseldorf, Germany
Ulrich Germing, MD

Johannes Gutenberg University Medical Center Mainz
Mainz, Germany
Markus Radsak, MD, PhD

Johann Wolfgang Goethe Universität
Frankfurt Main, Germany
Gesine Bug, MD

Klinikum Rechts der Isar Technical University of Munich
Munich, Germany
Katharina Götze, MD

MLL Münchner Leukämielabor
Munich, Germany
Torsten Haferlach, MD
Wolfgang Kern, MD

Rems-Murr-Klinik Winnenden
Winnenden, Germany
Prof. Dr. med Markus Schaich, MD

St. Johannes Hospital Heinrich-Heine Universität
Duisburg, Germany
Carlo Aul, MD,PhD
Aristotle Giagounidis, MD, PhD

Albert-Ludwigs-Universität Freiburg
Freiburg, Germany
Michael Lübbert, MD, PhD

Universität Hamburg
Hamburg, Germany
Nicolaus Kröger, MD, PhD

Universitätsklinikum Carl Gustav Carus
Dresden, Germany
Katja Sockel, MD

University Children's Hospital
Freiburg, Germany
Charlotte Niemeyer, MD

University of Cologne
Cologne, Germany
Karl-Anton Kreuzer, MD

University Hospital Essen Dept. for Hematology and Stem Cell Transplantation
Essen, Germany
Thomas Schroeder, MD

University Hospital Leipzig
Leipzig, Germany
Uwe Platzbecker, MD

University Hospital Mannheim
Mannheim, Germany
Wolf-Karsten Hofmann, MD

GREECE

Patras University Hospital
Patras, Greece
Argiris Symeonidis, MD

University of Athens Laikon Hospital
Athens, Greece
Panagiotis Diamantopoulos MD, PhD

University General Hospital Attikon
Athens, Greece
Vassiliki Pappa, MD

HUNGARY

Semmelweis University School of Medicine
Budapest, Hungary
Judit Várkonyi, MD, PhD

INDIA

Tata Medical Centre
Kolkata, India
Col (Dr.) Deepak Kumar Mishra, MD

Tata Memorial Hospital
Mumbai, India
Manju Sengar, MD

IRELAND

Adelaide and Meath Hospital
Dublin, Ireland
Helen Enright, MD

ISRAEL

Tel-Aviv Sourasky Medical Center
Tel-Aviv, Israel
Moshe Mittelman, MD

Chaim Sheba Medical Center
Tel Hashomer, Israel
Drorit Grizim Merkel, MD

ITALY

Cancer Center – IRCCS Humanitas Research Hospital
Milan, Italy
Matteo G. Della Porta, MD

Centro di Riferimento Oncologico di Basilicata (CROB)
Rionero in Vulture (PZ), Italy
Pellegrino Musto, MD

Istituto di Ematologia Universita' Cattolica Sacro Cuore
Rome, Italy
Giuseppe Leone, MD

Policlinico Universitario di Roma Tor Vergata
Rome, Italy
Sergio Amadori, MD
Maria Teresa Voso, MD

S. Eugenio Hospital Tor Vergata University
Rome, Italy
Paolo de Fabritiis, MD
Pasquale Niscola, MD

University of Florence Azienda OSP Careggi
Florence, Italy
Valeria Santini, MD

University of Pavia School of Medicine Fondazione IRCCS Policlinico San Matteo
Pavia, Italy
Mario Cazzola, MD

JAPAN

Kyoto University Hospital
Kyoto, Japan
Akifumi Takaori, MD

Metropolitan Research and Treatment Center for Blood Disorders (MRTC Japan)
Tokyo, Japan
Kiyoyuki Ogata, MD, FACP

Nagasaki University Hospital School of Medicine, Atomic Bomb Disease Institute
Nagasaki City, Japan
Yasushi Miyazaki, MD

Saitama Medical University International Medical Center
Hidaka, Saitama, Japan
Tomoya Maeda, MD, PhD

Tokyo Medical College
Tokyo, Japan
Kazuma Ohyashiki, MD, PhD

MEXICO

Instituto Nacional de Ciencias Medicas y Nutrición Salvador Zubiran American-British-Cowdray Cancer Center
Mexico City, Mexico
Alvaro Aguayo, MD

THE NETHERLANDS

Radboud University Nijmegen Medical Center
Nijmegen, The Netherlands
Saskia M.C. Langemeijer, MD

Vrije Universiteit Medical Center
Amsterdam, The Netherlands
Arjan A. van de Loosdrecht, MD, PhD

MDS Foundation Centers of Excellence

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

NORWAY

Haukeland University Hospital
Bergen, Norway
Astrid Olsnes Kittang, MD, PhD

POLAND

Jagiellonian University Collegium Medicum
Kraków, Poland
Aleksander Skotnicki, MD, PhD

Medical University of Warsaw
Warsaw, Poland
Krzysztof Madry, MD

PORTUGAL

Hospital de Santa Maria
Lisbon, Portugal
Joao F. Lacerda, MD

SAUDI ARABIA

King Faisal Specialist Hospital & Research Centre
Riyadh, Saudi Arabia
Amr Hanbali, MD

King Khaled University Hospital King Saud University
Riyadh, Saudi Arabia
Ak Almomen, MD

SINGAPORE

Singapore General Hospital
Aloysius Ho, MD

SOUTH AFRICA

University of Cape Town Groote Schuur Hospital
Cape Town, South Africa
Nicolas Novitzky, MD, PhD

SOUTH KOREA

Catholic Blood and Marrow Transplantation Center The Catholic University of Korea
Seoul, Korea
Yoo-Jin Kim, MD

Seoul National University Hospital Seoul National University College of Medicine
Seoul, Korea
Dong Soon Lee, MD, PhD

Yonsei Cancer Centre, Severance Hospital Yonsei University College of Medicine
Seoul, Korea
June-Won Cheong, MD, PhD

SPAIN

Hospital Universitario de Salamanca
Salamanca, Spain
Maria Diez-Campelo, MD, PhD

Hospital Universitario La Fe
Valencia, Spain
Guillermo F. Sanz, MD, PhD

Vall d'Hebron Institute of Oncology Universitary Hospital Vall d´Hebron
Barcelona, Spain
David Valcárcel, MD, PhD

SWEDEN

Karolinska Institute at Karolinska University Hospital Huddinge
Stockholm, Sweden
Eva Hellström-Lindberg, MD, PhD

SWITZERLAND

Basel University Hospital
Basel, Switzerland
Jakob R. Passweg, MD, MS

Bern University Hospital and University of Bern
Bern, Switzerland
Nicolas Bonadies, MD

St. Claraspital AG
Basel, Switzerland
Stefani Parmentier, MD

University Hospital Zurich
Zurich, Switzerland
Markus G. Manz, MD
Stefan Balabanov, MD

TAIWAN

Chang Gung Memorial Hospital Chang Gung University
Taoyuan, Taiwan
Lee-Yung Shih, MD

National Taiwan University Hospital
Taipei, Taiwan
Hwei-Fang Tien, MD, PhD

THAILAND

King Chulalongkorn Memorial Hospital
Pathumwan, Bangkok, Thailand
Tanin Intragumtornchai, MD

TUNISIA

Hospital Aziza Othmana
Tunis, Tunisia
Balkis Meddeb, MD

TURKEY

Ankara University School of Medicine Hospital
Ankara, Turkey
Osman Ilhan, MD

UKRAINE

Research Center for Radiation Medicine
Kiev, Ukraine
Dimitry Bazyka, MD

UNITED KINGDOM

Aberdeen Royal Infirmary Aberdeen University School of Medicine
Foresterhill, Aberdeen, Scotland
Dominic Culligan, MD

Cambridge University Hospitals
Cambridge, United Kingdom
Alan J. Warren, MD, PhD

Christie NHS Foundation Trust
Manchester, United Kingdom
Mike Dennis, MD
Dan Wiseman, MD

King's College London & King's College Hospital
London, United Kingdom
Ghulam J. Mufti, DM, FRCP, FRCPath

Queen Elizabeth Hospital University Hospital Birmingham NHS Trust
Birmingham, United Kingdom
Manoj Raghavan, MD

Radcliffe Hospitals & University of Oxford
Oxford, United Kingdom
Paresh Vyas, MD

St. James's University Hospital St. James's Institute of Oncology
Leeds, United Kingdom
Catherine Cargo, MD

University Hospital Southampton (NHS Foundation Trust)
Southampton, Hampshire, UK
Christopher Dalley, MD
Srinivasan Narayanan, MD

University Hospital of Wales
Cardiff, Wales
Jonathan Kell, MD

VIETNAM

National Institute of Hematology and Blood Transfusion
Hanoi, Vietnam
Khanh Quoc Bach, MD, PhD

Servier Announces FDA Approval of TIBSOVO® (ivosidenib tablets) for the Treatment of IDH1-Mutated Relapsed or Refractory (R/R) Myelodysplastic Syndromes (MDS)

TIBSOVO is the first and only approved targeted therapy for R/R MDS patients with a susceptible IDH1 mutation

Fifth approved indication for TIBSOVO solidifies

BOSTON, MA — Oct. 24, 2023 (PRNewswire). Servier, a leader in oncology committed to bringing the promise of tomorrow to the patients we serve, today announced the U.S. Food and Drug Administration (FDA) has approved TIBSOVO® (ivosidenib tablets) for the treatment of patients with isocitrate dehydrogenase 1 (IDH1)-mutated relapsed or refractory (R/R) myelodysplastic syndromes (MDS). This is the fifth indication for TIBSOVO across IDH1-mutated cancers, and the first and only approved targeted therapy for people diagnosed with R/R MDS within this molecularly defined subset.

"Servier is proud to lead the way in mutant IDH inhibition through continued innovations that support patients living with difficult and hard-to-treat cancers," said Arjun Prasad, Head of Commercial, Servier Pharmaceuticals. "As the first and only targeted therapy available for patients with IDH1-mutated relapsed or refractory myelodysplastic syndromes, today's FDA approval for TIBSOVO reinforces our commitment to deliver significant advances in areas of high unmet need and bring the right treatment, to the right patient, at the right time."

The FDA approval of this indication is supported by a pivotal Phase 1, open-label study in IDH1-mutated R/R MDS patients (n=18) where a complete remission (CR) rate of 38.9% and objective response rate (ORR) of 83.3% were documented in patients treated with TIBSOVO. In addition, the median time to CR was 1.9 months (range: 1.0, 5.6). At the time of data cutoff, the median duration of CR had not been reached (range: 1.9, 80.8+*) and the median overall survival was 35.7 months (range: 3.7*, 88.7*). Additionally, of the nine patients who were transfusion dependent with red blood cells or platelets at baseline, 66.7% (n=6) became independent of transfusions during any ≥56-day post-baseline period. Overall, treatment-related adverse events were consistent with the known safety profile of TIBSOVO.

"The novel use of targeted therapy across IDH-mutated cancers has become a powerful therapeutic option for patients within this molecularly defined subset," said Amir Fathi, M.D., hematologist, medical oncologist, and expert in myeloid malignancies. "This new indication in IDH1-mutated relapsed or refractory myelodysplastic syndromes reinforces the importance of mutational testing to inform treatment decisions and potentially improve patient outcomes."

An estimated 16,000 people in the U.S. are diagnosed with MDS each year. Approximately 3.6% of MDS patients have an IDH1 mutation, which is considered an early "driver" mutation. For MDS patients with an IDH1 mutation, the prognosis has often been associated with worse overall outcomes and an increased risk of transformation to AML.

"This approval for TIBSOVO is welcome news for the MDS community," said Tracey Iraca, Executive Director, MDS Foundation. "Before today, there were no approved targeted therapies available to relapsed or refractory MDS patients harboring the IDH1-mutation. We want to thank the study participants, their families and caregivers, as well as the researchers at Servier and clinical investigators involved in this study for helping to bring a new treatment option to patients where there has been a significant unmet need."

"An MDS diagnosis is ambiguous. I remember feeling confused trying to make sense of my diagnosis, what having an IDH1-mutation meant, and what options were available for my treatment plan," said Susan, a patient living with IDH1-mutated MDS.** "The news of an FDA approval for a targeted therapy in IDH-1 mutated MDS has given me a tremendous sense of gratitude and provides hope to patients – like me – who are living with this disease. I want to thank everyone who played a role in this major step forward for the MDS community."

TIBSOVO was granted Breakthrough Therapy designation for the treatment of adult patients with R/R (MDS) with an IDH1 mutation and received Priority Review, which accelerated the review timeline and is granted to applications for medicines that, if approved, would provide significant improvements in the effectiveness or safety of the treatment, diagnosis, or prevention of serious conditions.

The FDA also approved the Abbott RealTime IDH1 Assay as a companion diagnostic device to select patients for TIBSOVO.

* Denotes a censored observation. **Last name withheld to protect privacy.

ABOUT THE NCT02074839 CLINICAL TRIAL

This Phase I, open-label multinational study evaluated the safety, tolerability, and clinical activity of ivosidenib in patients with relapsed or refractory myelodysplastic syndromes with an IDH1 mutation. The primary endpoint was complete remission (CR) plus partial remission (PR) rate and key secondary endpoints included duration of CR+PR, duration of transfusion independence, and time to transfusion independence.

ABOUT TIBSOVO® (IVOSIDENIB TABLETS)

TIBSOVO is a precision medicine that targets a specific type of mutation known as isocitrate dehydrogenase 1 (IDH1). TIBSOVO is approved in five indications globally, including approvals in the U.S., European Union, Australia, and China.

In the U.S., TIBSOVO is approved for the treatment of adults with IDH1-mutant relapsed or refractory AML and in monotherapy or in combination with azacitidine for adults with newly diagnosed IDH1-mutant AML who are ≥75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy, as monotherapy for the treatment of adult patients with IDH1-mutant relapsed or refractory MDS, and for patients with previously treated IDH1-mutated cholangiocarcinoma.

Servier has granted CStone a co-exclusive license for the development and an exclusive license agreement for the commercialization of TIBSOVO in Mainland China, Taiwan, Hong Kong, Macao and Singapore.

For more information about TIBSOVO in the U.S., please visit www.tibsovo.com.

Geron Announces FDA Acceptance of New Drug Application for Imetelstat for the Treatment of Lower Risk MDS

FOSTER CITY, CA — August 21, 2023 (BUSINESS WIRE).
Geron Corporation (Nasdaq: GERN), a late-stage clinical biopharmaceutical company, today announced that the United States Food and Drug Administration (FDA) has accepted the filing of Geron's New Drug Application (NDA) for imetelstat, its first-in-class telomerase inhibitor, for the treatment of transfusion-dependent anemia in patients with lower risk myelodysplastic syndromes (MDS).

"The FDA's acceptance of our New Drug Application is an important landmark along our steadfast journey to bring telomerase inhibition with imetelstat to the market," said John A. Scarlett, M.D., Geron's Chairman and Chief Executive Officer. "We look forward to continuing our collaboration with the FDA toward the goal of bringing imetelstat to the many patients for whom we believe this treatment could make a significant difference."

"FDA acceptance of our NDA is a significant milestone for both Geron and the MDS community, as there remain few treatment options and significant unmet needs, particularly for patients with difficult-to-treat subtypes of this cancer," said Faye Feller, M.D., Executive Vice President, Geron's Chief Medical Officer. "We believe that the IMerge Phase 3 data reflect the truly unique attributes of imetelstat, and, if approved, we expect imetelstat will change the standard of care in lower risk MDS."

The NDA submission is based on results from IMerge Phase 3, in which the primary endpoint of 8-week transfusion independence (TI) was significantly higher with imetelstat vs. placebo ($p<0.001$), with median TI duration approaching one year for imetelstat 8-week TI responders. Mean hemoglobin levels in imetelstat-treated patients increased significantly ($p<0.001$) over time compared to placebo patients. Statistically significant and clinically meaningful efficacy results were achieved across key MDS subgroups irrespective of ring sideroblast (RS) status, baseline transfusion burden and IPSS risk category. Patient-reported outcomes (PRO) data reported a sustained meaningful improvement in fatigue for imetelstat-treated patients vs. placebo. Safety results were consistent with prior imetelstat clinical experience.

As allowed under the 21st Century Cures Act, the FDA has up to 74 days from the NDA submission date to notify Geron of the Prescription Drug User Fee Act (PDUFA) action date for the NDA. Upon receipt of this notification, Geron plans to disclose the timeline for the NDA review.

Additionally, Geron expects to submit a Marketing Authorization Application (MAA) in the European Union (EU) in the fourth quarter of 2023.

ABOUT IMERGE PHASE 3

The Phase 3 portion of the IMerge Phase 2/3 study is a double-blind, 2:1 randomized, placebo-controlled clinical trial to evaluate imetelstat in patients with IPSS Low or Intermediate-1 risk (lower risk) transfusion dependent MDS who were relapsed after, refractory to, or ineligible for, erythropoiesis stimulating agent (ESA) treatment, had not received prior treatment with either a HMA or lenalidomide and were non-del(5q). To be eligible for IMerge Phase 3, patients were required to be transfusion dependent, defined as requiring at least four units of packed red blood cells (RBCs), over an eight-week period during the 16 weeks prior to entry into the trial. The primary efficacy endpoint of IMerge Phase 3 is the rate of red blood cell transfusion independence (RBC-TI) lasting at least eight weeks, defined as the proportion of patients without any RBC transfusion for at least eight consecutive weeks since entry to the trial (8-week TI). Key secondary endpoints include the rate of RBC-TI lasting at least 24 weeks (24-week TI), the duration of TI and the rate of hematologic improvement erythroid (HI-E), which is defined under 2006 IWG criteria as a rise in hemoglobin of at least 1.5 g/dL above the pretreatment level for at least eight weeks or a reduction of at least four units of RBC transfusions over eight weeks compared with the prior RBC transfusion burden. A total of 178 patients were enrolled in IMerge Phase 3 across North America, Europe, Middle East and Asia.

ABOUT IMETELSTAT

Imetelstat is a novel, first-in-class telomerase inhibitor exclusively owned by Geron and being developed in hematologic malignancies. Data from non-clinical studies and clinical trials of imetelstat provide strong evidence that imetelstat targets telomerase to inhibit the uncontrolled proliferation of malignant stem and progenitor cells in myeloid hematologic malignancies resulting in malignant cell apoptosis and potential disease-modifying activity. Imetelstat has been granted Fast Track designation by the U.S. Food and Drug Administration for both the treatment of adult patients with transfusion dependent anemia due to Low or Intermediate-1 risk MDS that is not associated with del(5q) who are refractory or resistant to an erythropoiesis stimulating agent, and for adult patients with Intermediate-2 or High-risk MF whose disease has relapsed after or is refractory to janus associated kinase (JAK) inhibitor treatment. Imetelstat is currently not approved by any regulatory authority.

COMMANDS Trial: First-Line Luspatercept Boosts Chance of Transfusion Independence in Lower-Risk MDS

June 10, 2023. In the global phase III COMMANDS trial of patients with low-risk transfusion-dependent myelodysplastic syndrome (MDS), with or without ring sideroblasts, treatment with luspatercept essentially doubled the likelihood of achieving transfusion independence and an increase in hemoglobin level, compared with epoetin alfa, an erythropoiesis-stimulating agent (ESA), in a planned interim analysis.[1]

"Luspatercept is the first and only therapy to demonstrate superiority in a head-to-head study against ESAs in transfusion-dependent lower-risk MDS. This is important, as ESAs have been the first-line treatment for patients with lower-risk MDS for decades," said Guillermo Garcia-Manero, MD, Professor in the Department of Leukemia and Chief of the Section of Myelodysplastic Syndromes at The University of Texas MD Anderson Cancer Center in Houston.

Luspatercept, an erythroid maturation agent, is approved by the U.S. Food and Drug Administration for the treatment of patients with lower-risk MDS who meet certain criteria, including the presence of ring sideroblasts and failure on (or ineligibility for) an ESA, based on the results of the MEDALIST trial.[2]

The subsequent global COMMANDS trial evaluated the drug in a wider population that included patients with and without ring sideroblasts. The primary endpoint was transfusion independence for at least 12 weeks within the first 24 weeks with a concurrent mean hemoglobin increase of at least 1.5 g/dL. At the time of this planned interim analysis (representing 80% of participants), 58.5% receiving luspatercept met this endpoint, compared with 31.2% receiving epoetin alfa (P<.0001), Dr. Garcia-Manero reported in a press briefing prior to the 2023 ASCO Annual Meeting.

He maintained that COMMANDS would usher in a "paradigm shift" in the treatment of patients with lower-risk MDS-associated anemia who are naive to prior therapy. "I think that's going to happen with the presentation of these results…. They will move traditional ESAs over and establish luspatercept as front-line therapy."

COMMANDS DETAILS

The global phase III COMMANDS clinical trial included 354 patients with lower-risk MDS as defined by the revised International Prognostic Scoring System (IPSS-R) criteria. Patients were ESA-naïve, had less than 5% bone marrow blasts and serum EPO levels less than 500 U/L, and required red blood cell transfusions (defined as 2–6 units 8 weeks for at least 8 weeks immediately prior to randomization).

Patients were randomly assigned to receive subcutaneous luspatercept (starting dose 1.0 mg/kg, titration up to 1.75 mg/kg) once every 3 weeks for at least 24 weeks (n = 178) or epoetin alfa (starting dose 450 IU/kg, titration up to 1,050 IU/kg) once a week for at least 24 weeks (n = 176). At the planned interim analysis of 301 patients, the median treatment duration was 41.6 weeks for luspatercept and 27.0 weeks for epoetin alfa.

OUTCOMES BY RING SIDEROBLAST STATUS

"For the primary endpoint, patients receiving luspatercept, regardless of subgroup, achieved transfusion independence with a hemoglobin increase," Dr. Garcia-Manero said. The drug was effective regardless of baseline serum erythropoietin level, red blood cell transfusion burden, SF3B1 mutation status, or ring sideroblast status.

For patients with ring sideroblasts (n = 220), the primary endpoint was met by 64.8% receiving luspatercept vs 25.9% receiving the ESA. For the ring sideroblast–negative subset (n = 80), this endpoint was met by 41.0% vs 46.3%.

Dr. Garcia-Manero said the disproportionate number of patients with ring sideroblasts was the result of the more common occurrence of this phenotype and the approval of luspatercept in ring sideroblast–positive patients. "The study was not powered to see a significant difference between ESAs and luspatercept in the ring sideroblast–negative context, but the results are not inferior. Actually, they are quite similar," he said. "Furthermore, look at the duration of response…. Patients receiving luspatercept experienced longer durations of transfusion independence, regardless of ring sideroblast status."

Median durations of transfusion independence with luspatercept vs epoetin alfa, with hazard ratios (HR) and 95% confidence intervals, follow: all patients, 126.6 vs 77.0 weeks (HR = 0.456 [CI = 0.260–0.798]); ring sideroblast–positive, 120.9 vs 47.0 weeks (HR = 0.626 [CI = 0.361–1.085]); ring sideroblast–negative: not estimable vs 95.1 weeks (HR = 0.492 [CI = 0.148–1.638]).

SECONDARY ENDPOINTS AND TOLERABILITY

The secondary endpoints in the study included hematologic improvement–erythroid response ≥8 weeks and red blood cell transfusion independence at 24 weeks and at ≥12 weeks. Across these endpoints, as with the primary endpoint, luspatercept was more effective than epoetin alfa (**Table 1**), Dr. Garcia-Manero reported.

Safety was consistent with previous experience with luspatercept, according to Dr. Garcia-Manero. Adverse events were slightly more common with luspatercept (92.1%) than epoetin alfa (85.2%), as were those considered related to treatment (30.3% vs 17.6%). Treatment discontinuation occurred in 4.5% and 2.3%, respectively.

The most common grade 3 or 4 adverse events attributed to treatment with luspatercept were anemia (7.3%), thrombocytopenia (3.9%), neutropenia (3.9%), and dyspnea (3.9%). Transformation to acute myeloid leukemia was observed in 2.2% of the luspatercept arm and 2.8% of the epoetin arm. All-cause mortality was similar, 18% in each arm.

Dr. Garcia-Manero said the favorable toxicity profile, coupled with the ease of administration (every 3 weeks), "is probably why this drug, in my opinion, will likely become the standard of care for our patients with lower-risk MDS, whether they are ring sideroblast–positive or –negative. I think there will be a paradigm shift for most patients."

MDS Foundation
The Myelodysplastic Syndromes Foundation, Inc.

TABLE 1. Outcomes with Luspatercept vs Epoetin Alfa

ENDPOINT	LUSPATERCEPT	EPOETIN ALFA	*P* VALUE
Transfusion independence ≥12 weeks + hemoglobin increase*	58.5%	31.2%	*P* < 0.0001
HI-E response	74.1%	51.3%	*P* < 0.0001
Transfusion independence ≥24 weeks	47.6%	29.2%	Not applicable
Transfusion independence ≥12 weeks	66.7%	46.1%	Not applicable

*≥1.5 g/dL within the first 24 weeks.
HI-E=hematologic-erythroid improvement ≥8 weeks

DISCLOSURE: The study was funded by Bristol Myers Squibb. Dr. Garcia-Manero reported financial relationships with AbbVie, Acceleron Pharma, Astex Pharmaceuticals, Bristol Myers Squibb/Celgene, Curis, Genentech, Gilead Sciences, and Novartis.

REFERENCES

1. Garcia-Manero G, Platzbecker U, Santini V, et al: Efficacy and safety results from the COMMANDS trial. 2023 ASCO Annual Meeting. Abstract 7003. Presented at a press briefing May 22, 2023. 2. Fenaux P, Platzbecker U, Mufti GJ, et al: Luspatercept in patients with lower-risk myelodysplastic syndromes. *N Engl J Med* 382:140–151, 2020.

MDSF Quarterly Interaction Metrics

In an effort to mathematically quantify our outreach efforts, the MDSF will now be distributing quarterly interaction metrics.

The staff at the MDS Foundation works hard to support patients and their caregivers. We work to find answers, supply educational resources, provide emotional support, and connect patients with our MDS Centers of Excellence.

The data provided has been pulled from phone inquiries, email communications, and message board posts. Patients, family & friends, and members of the community find us primarily through internet searches. The other two more commonly seen referral sources are our Centers of Excellence partners and word-of-mouth of others who have called in previously.

Although the MDSF serves as an advocate for all who reach out, we know there are still people we are missing. In our *Building Blocks of Hope* publication, we encourage patients to become a partner in their care. In order to do this, patients need to be fully informed on their disease and medical advances over time. As the Director of Patient Care, the highlight of my day is speaking with those impacted by MDS and helping them to decipher the available information. Please refer anyone you feel may benefit from our services to the MDSF. Contact information is listed below. Next quarterly metrics will be reviewed ~January 19, 2024.

Centers of Excellence Referrals

Dana-Farber (2); Duke (1); Fred Hutchinson (2); Johns Hopkins (1); Mayo Florida (1); Mayo Rochester (1); MD Anderson (2); Memorial Sloan-Kettering (1); Mount Sinai (1); Policlinico Tor Vergata (1); Stanford (1); Texas Oncology Austin (1); University of Alabama (1); University of Arizona (1); University of Southern California (2); University of Kansas (1); Vanderbilt (3)

Total Referrals: 23 (24.7% of those who reached out to the MDSF)

Snapshot of Inquiries

Question Type	No. and % of Inquiries from Those who Reached Out
Generalized MDS questions	40 (43%)
Looking for education resources	27 (29%)
Discussed clinical trials	16 (17.2%)
Seeking information on webinars/forums/events	6 (6.4%)
Questions requiring provider input	6 (6.4%)
Needing financial resources	6 (6.4%)
Fundraising and donations	2 (2.1%)

Ashley Moncrief, RN, BSN, Director of Patient Care
1-800-637-0839 ext 210, amoncrief@mds-foundation.org

METRICS

13-WEEK SUMMARY
7/24/23 – 10/21/23

No. of unique patients/ caregivers who called in: **54**

No. of unique patients/ caregivers who emailed: **26**

Message boards answered: **10**

Forum Follow-Ups: **3**

AS OF 10/21/23:

Assisted **93** different patients/caregivers/friends in 13 weeks (~7 patients per week)

SUPPORT GROUPS

have been requested in the following locations:*

SACRAMENTO, CALIFORNIA • HAWAII • NEW YORK • NEW JERSEY • KANSAS • TEXAS (Northwest & Fort Worth) • VIRGINIA BEACH, VIRGINIA

**Plus more virtual groups*

Advancing Research & Patient Care

The 18th International Congress on

Myelodysplastic Syndromes

Rotterdam, The Netherlands
7–10 May 2025

Join international MDS experts to learn about the latest advances, discoveries, basic and translational research, and MDS diagnosis, prognosis, and management.

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