



Autologous Stem Cell Transplantation (ASCT) 101

April 24, 2024

Tech Support

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Advancing MRD measurement.
Empowering patient care.



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Resources

- Resource tab includes
 - Speaker bios
 - Copy of the slide presentation
 - Exhibit Hall

**Submit your questions
throughout the program!**

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MMRF Research Initiatives

1. MMRF Myeloma Accelerator Challenge (MAC) Grants

- Broad, multi-institutional research grants designed to advance clinical trial concepts in the areas of
 - High-risk newly diagnosed multiple myeloma (NDMM)
 - High-risk smoldering myeloma (SMM)
- Each research network will be funded up to \$10M over 3 years

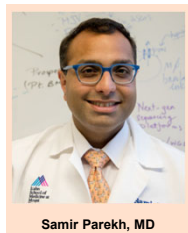
2. MMRF Horizon Adaptive Platform Trials

- Paired with MAC grants
- Done in collaboration with 13 MMRC sites
- Trials in relapsed/refractory myeloma, high-risk NDMM, high-risk SMM

For more information, visit themmrf.org

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2023 Myeloma Accelerator Challenge Program Grant Recipients



Samir Parekh, MD

Transforming Treatment of High-Risk Myeloma

Network includes Tisch Cancer Center at Mt Sinai, Albert Einstein Medical College, Hackensack University Medical Center, Stanford University Medical Center, UCSF, Washington University of Saint Louis



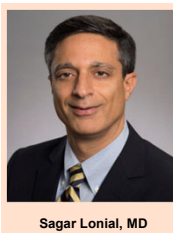
Pieter Sonneveld, MD, PhD

A Systems Biology Approach to High-Risk Myeloma

Network includes Erasmus Medical Center, Rotterdam; Amsterdam University Medical Centers; Julius Maximilian University of Wurzburg; University of Turin; University of Salamanca



Each network will receive \$7M over 3 years for a total \$21M investment by the MMRF, meant to foster collaboration and advance compelling hypotheses that are ready for rapid testing in clinical trials.



Sagar Lonial, MD

Clinical and Multi-Omics Platforms to Define High-Risk Smoldering Myeloma

Network includes Emory University, Atrium Health Levine Cancer Institute, Icahn School of Medicine at Mt. Sinai, Mass General Hospital, Mayo Clinic, MSKC Institute, Dana-Farber Cancer Institute

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Speakers

Sergio A. Giralt, MD, FACP, FASTCT

Memorial Sloan Kettering Cancer Center
Weill Cornell Medical College
New York, New York

David H. Vesole, MD, PhD, FACP

MedStar Georgetown University Hospital
Georgetown University School of Medicine
Washington, DC
John Theurer Cancer Center,
Hackensack Meridian School of Medicine
Hackensack, New Jersey



Deciding on an Autologous Stem Cell Transplant

Sergio A. Giralt, MD, FACP

Memorial Sloan Kettering Cancer Center
New York, New York

What is an Autologous Stem Cell Transplantation and Why is it a Part of Treatment for Myeloma?

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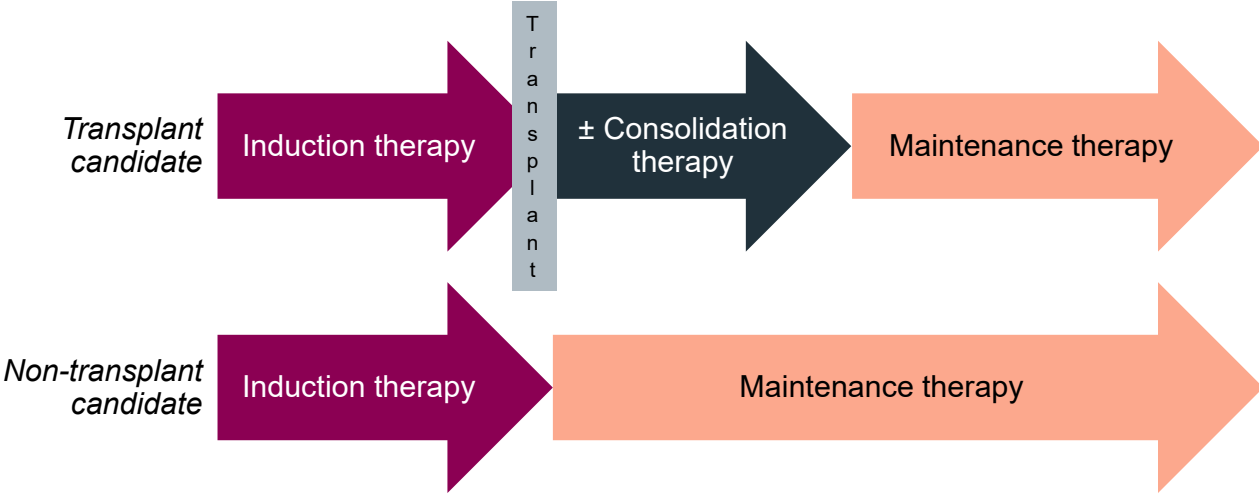
Types of Stem Cell Transplantation

Transplant type	Stem cell source	Transplant process
<ul style="list-style-type: none"> • Autologous* <ul style="list-style-type: none"> – Your own cells • Allogeneic <ul style="list-style-type: none"> – A donor’s cells (requires a match) 	<ul style="list-style-type: none"> • Autologous <ul style="list-style-type: none"> – Peripheral blood (99%) • Allogeneic <ul style="list-style-type: none"> – Peripheral blood – Bone marrow – Cord Blood 	<ul style="list-style-type: none"> • Autologous <ul style="list-style-type: none"> – Single – Tandem • Allogeneic <ul style="list-style-type: none"> – Myeloablative – Reduced Intensity

*Most common

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Current Treatment Paradigm for Newly Diagnosed Multiple Myeloma



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Who is a Candidate for Autologous Stem Cell Transplantation?

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Candidates for ASCT

- A patient's eligibility for high-dose chemotherapy and transplant is based on overall health.
- A variety of factors such as comorbid conditions, fitness, and frailty influence whether you are eligible for ASCT.
- Guidelines for patient eligibility may vary between cancer centers.

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What are the Pros and Cons Associated with Autologous Stem Cell Transplant?

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Pros and Cons of Autologous Stem Cell Transplant

Pros

- Track record of effectiveness
- Time off intensive therapy
- Able to repeat the procedure
- Low dose maintenance post-transplant therapy extends disease control even further

Cons

- Toxicity/long-term side effects
- Confinement in hospital/clinic
- Time away from home and work

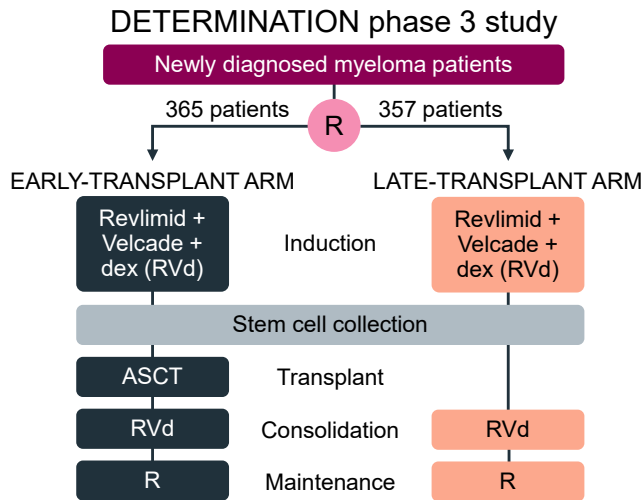
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Should I Do My Transplant Early or
Should I Delay My Transplant After First
Relapse?

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Early Versus Delayed Transplant?

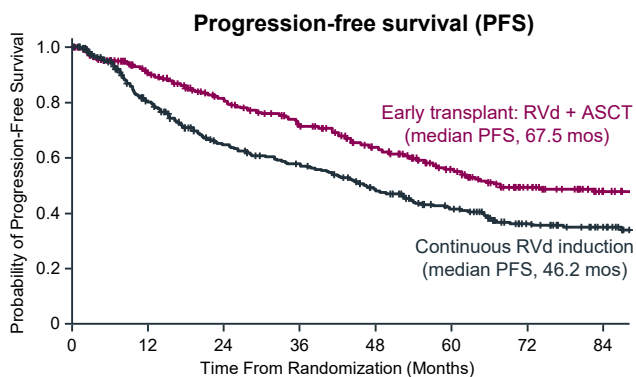
Q: Should I get a transplant after induction OR wait until relapse?



Richardson PG et al. *N Engl J Med.* 2022;387:132.

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Phase 3 Study of ASCT for Newly Diagnosed Multiple Myeloma: Survival Analysis



Transplant extended time to progression by 20 months

PFS for early transplant: approximately 5.5 years
PFS for continuous induction: approximately 4 years

Length of overall survival: no difference.

Richardson PG et al. *N Engl J Med.* 2022;387:132.

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Phase 3 Study of ASCT for Newly Diagnosed Multiple Myeloma: Side Effects

Side effect (%)	RVd alone (N=357)	RVd + ASCT (N=365)
Any	78.2	94.2
Low blood counts	60.5	89.9
Very low white cell count	42.6	86.3
Low platelet count	19.9	82.7
Low white cell count	19.6	39.7
Anemia	18.2	29.6
Lymphopenia	9.0	10.1
Infections with low WBC	4.2	9.0
Fever	2.0	5.2
Pneumonia	5.0	9.0
Diarrhea	3.9	4.9
Nausea	0.6	6.6
Mouth sores	0	5.2
Fatigue	2.8	6.0
Numbness, tingling nerve	5.6	7.1

Severe side effects were more common with transplant.

Richardson PG et al. *J Clin Oncol*. 2022;40. Abstract LBA4. Richardson PG et al. *N Engl J Med*. 2022;387:132.

Early vs Delayed Transplant: Pros and Cons



Pros

Early ASCT

- Deeper and more durable response
- Youngest/healthiest you are going to be
- Allows for fewer cycles of induction treatment

Late ASCT

- PFS may be shorter, but currently appears OS is the same
- Less side effects without high-dose chemotherapy
- Conserve quality of life in the early part of disease journey



Cons

Early ASCT

- No proven impact on overall survival
- 20% of patients still relapse within 2 years
- More side effects including a small risk of serious life-threatening complications
- 3 months to full clinical recovery

Late ASCT

- Need more cycles of induction
- May need next treatment sooner, including (late) transplant
- Not all patients relapsing are able to undergo salvage ASCT

Deciding on an ASCT

Summary

- ASCT remains the standard of care for frontline therapy of myeloma.
- ASCT safety has been established and it induces long PFS.
- Decision of ASCT should be individualized in every patient and deserves a thorough discussion between the patient and provider.
- Serves as a platform for new therapies.

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Undergoing an Autologous Stem Cell Transplant

David H. Vesole, MD, PhD, FACP

Georgetown University School of Medicine

Washington, District of Columbia

John Theurer Cancer Center

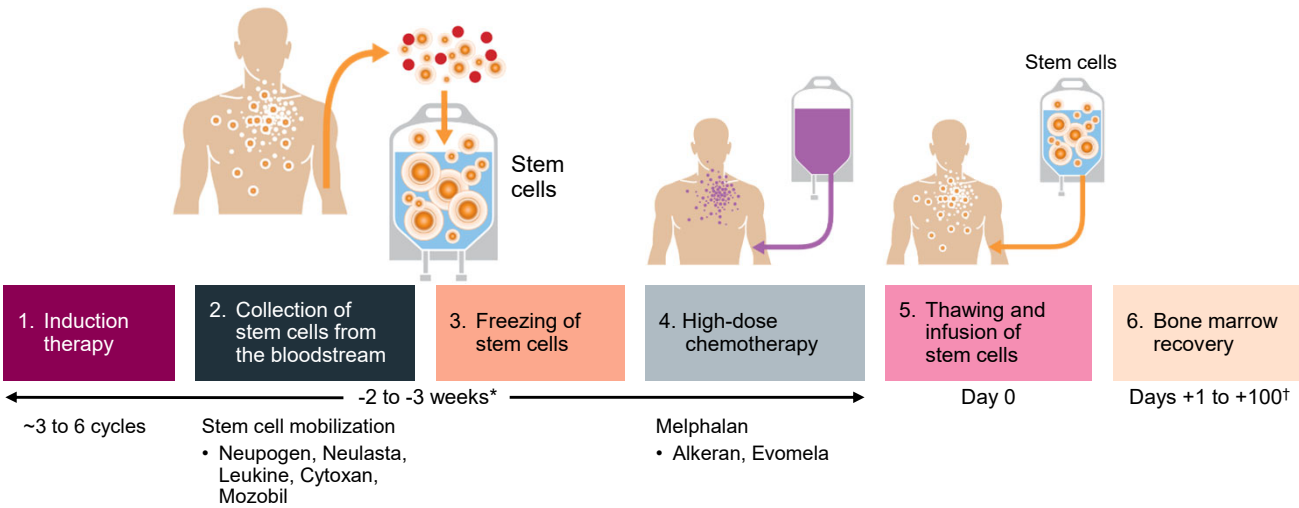
Hackensack, New Jersey

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What is Involved with Stem Cell Collection?

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Autologous Stem Cell Transplantation



*The weeks leading up to the transplant; †The days after the transplant.

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The Transplant Process

Outpatient (Clinic) Setting

Benefits

- Allows patients to spend nights in the comfort of their own home or on-campus housing
- The same health care team oversees both inpatient and outpatient programs

Process

- Daily visits to the infusion center
- Labs are drawn, infusions and IVs administered
- Patients are in a private treatment bay
- Typically spend 5–6 hours a day at the center
- Goal is to keep the patient from being hospitalized!

Caution

- Fever will require hospitalization; it is easy to admit a patient from the infusion center or home to the inpatient unit

Inpatient (Hospital) Setting

- Traditional way of administering high-dose chemotherapy with stem cell rescue
- Better option for some patients
- Hospitals typically offer care across a broad range of medical issues whereas clinics may offer a more focused and specialized approach to cancer treatment
- Age is no longer an issue; comorbidities play more of a role in determining whether or not to have your transplant inpatient or outpatient
- The inability to have a caregiver with a patient 24/7 is also a deciding factor in this decision

How Do I Prepare for Autologous Stem Cell Transplant?

Questions to ask your doctor about stem cell transplantation.

- Am I a candidate for high-dose chemotherapy and stem cell transplantation?
- When is the best time for me to undergo transplantation?
- Does your center do stem cell transplants?
- How many transplants has your center performed in multiple myeloma in the last year?
- Is the procedure performed as an inpatient or outpatient?
- How long will I be in the hospital?
- What kind of changes in my lifestyle will I need to make?
- When do I go back to you for follow-up?



Can I use my Stem Cells for CAR T-cell Therapy?

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The Difference Between Autologous Stem Cell Transplant and CAR T-cell Therapy

	Autologous stem cell transplantation	CAR T-cell therapy
Patient's cells collected	Yes	Yes
Types of cells collected	Stem cells [†]	T cells*
Collected cells are engineered in a lab	No	Yes
Patient given chemotherapy before cells are infused back into patient	Yes (melphalan)	Yes (lymphodepletion therapy)
Total time before cells are reinfused	~1 week	~3-4 weeks
Currently approved by the FDA for the treatment of MM	Yes	Yes
Side effects of treatment	Fatigue, nausea and vomiting, diarrhea, mouth sores, increased risk of infection, and low blood counts	Cytokine release syndrome, neurotoxicity, low blood counts, risk of infection, fatigue, nausea and vomiting, and diarrhea

*A type of white blood cell that can identify, target, and attack MM cells

[†]Precursor cells that give rise to many types of blood cells

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What Should I Expect After Stem Cell Transplant?

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After the Transplant: The Road to Recovery

<p>Rest</p> 	<p>Improved nutrition</p> 	<p>Exercise</p> 	<p>Infection prevention</p> 
 <p>Antivirals and antibiotics for 6–12 months after treatment</p>	 <p>Avoid crowds</p>	 <p>Ongoing follow-up with your doctor</p>	 <p>Restage myeloma 100 days after transplant</p>

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Post-Transplant Maintenance Therapy Options

	Preferred	Other recommended regimens	Useful in certain circumstances
Transplant eligible	<ul style="list-style-type: none"> • Revlimid* 	<ul style="list-style-type: none"> • Velcade 	<ul style="list-style-type: none"> • Velcade-Revlimid ± dex[#] • Kyprolis-Revlimid • Darzalex ± Revlimid • Ixazomib[†]

*Category 1 recommendation. Based on high-level evidence, there is uniform NCCN consensus that the intervention is appropriate.
[#]Two drug maintenance recommended for high-risk MM.
[†]Ixazomib may be substituted for carfilzomib in select patients.

National Comprehensive Cancer Network Guidelines Version 3.2024. Multiple Myeloma.

What Vaccinations are Required Following ASCT, and When?

Vaccination Record Name _____

Vaccine	Start Date	# of Doses	Date Given	Initials
Inactivated Influenza	4 to 6 months	1		
Tetanus/Diphtheria/Acellular Pertussis (Tdap)	12 months	4 to 5		
Haemophilus Infl. Conjugate (Hib)	12 months	4 to 5		
Inactivated Polio	12 months	4 to 5		

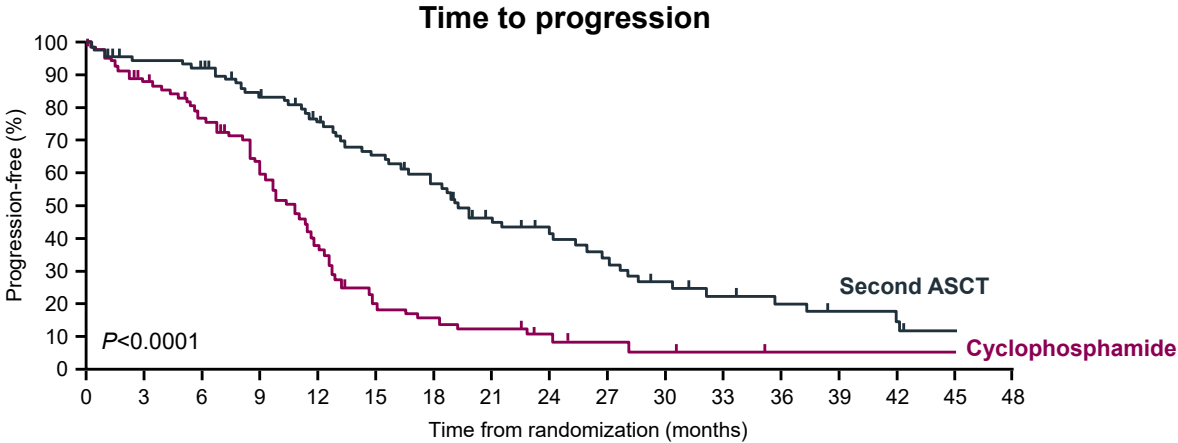
Vaccine	Start Date	# of Doses	Date Given	Initials
Pneumococcal conjugate (PCV)	12 months	4 to 5		
Hepatitis A and B (Recombinant)	12 months	3		
Zoster Vaccine Recombinant	12 months	2		
Measles/Mumps/Rubella	24 months	1 to 2		

Some centers do not vaccinate patients if they are on active treatment or have not recovered B-cell production. Good practice to ascertain that vaccination has worked (ie, check titers).

Will I Have to Go Through Another Transplant if I Relapse?

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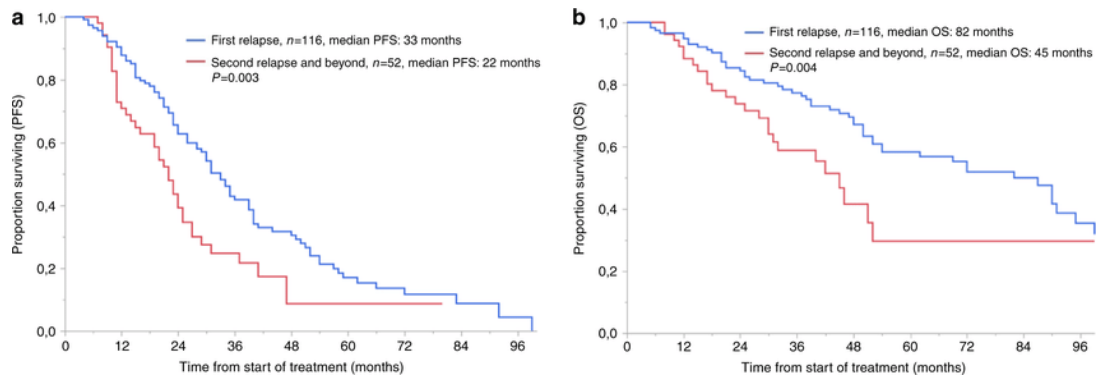
Second ASCT an Option for Early Relapse



Cook G et al. *Lancet Oncol.* 2014;15:874.

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Outcomes of ASCT in Patients with RRMM



Lemieux C et al. *Bone Marrow Transplant*. 2021;56(11):2664-2671.

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Undergoing an ASCT *Summary*

- High-dose chemotherapy with ASCT (if you are eligible) offers the best chance for long-lasting remission.
- You can undergo ASCT as an inpatient (you stay in the hospital before, during, and immediately after the transplant) or an outpatient (you make daily visits to a clinic) procedure.
- Second ASCT may be an option for some patients

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Questions & Answers

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Multiple Myeloma High-Impact Topic
MINIMAL RESIDUAL DISEASE (MRD)

Multiple Myeloma High-Impact Topic
BISPECIFIC ANTIBODIES

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Multiple Myeloma High-Impact Topic
THE RIGHT TRACK

Multiple Myeloma High-Impact Topic
MAINTENANCE THERAPY

MMRF MULTIPLE MYELOMA Research Foundation

Check out our **High-Impact Topic** VIDEOS

For more information, visit themmrf.org/educational-resources/

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MMRF Patient Resources

EXPECT GUIDANCE.

MMRF Patient Navigation Center

- Information & Resources
- Expert Advice
- Support

MMRF MULTIPLE MYELOMA Research Foundation

MMRF Patient Navigation Center

You and your care team will have many decisions to make along your treatment journey. The Patient Navigation Center is a space for multiple myeloma patients and their caregivers to connect with patient navigators – who are professionals specializing in oncology – for guidance, information, and support. You can connect with a patient navigator via phone, or email. Whatever questions you may have, our patient navigators are here to help.

MMRF Patient Navigators include:

- Grace Allison, RN, BSN, OCN, RN-BC
- Brittany Hartmann, RN-BSN
- Erin Mensching, RN-BSN, OCN

THE RIGHT TRACK

Get on the right track for you

The MMRF's Right Track program puts you on the path to the best results for you.

<p>Right Team</p> <p>Access experts and centers that have extensive experience treating multiple myeloma.</p>	<p>Right Tests</p> <p>Get the information, tests, and precise diagnoses to make the right treatment decisions.</p>	<p>Right Treatment</p> <p>Work with your team to consider the best treatment plan and identify clinical trials that are right for you.</p>
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Contact the Patient Navigation Center Today

Looking for guidance? We're here to help.

Monday – Friday | 9:00am – 7:00pm ET

Phone: 1-888-841-MMRF (6673) Online: TheMMRF.org/PatientNavigationCenter

Email: patientnavigator@themmrf.org

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Myeloma Mentors®

Myeloma Mentors® allows patients and caregivers the opportunity to connect with trained mentors. This is a phone-based program offering an opportunity for a patient and/or caregiver to connect one-on-one with a trained patient and/or caregiver mentor to share his or her myeloma journeys and experiences.

No matter what your disease state—smoldering, newly diagnosed, or relapsed/refractory—our mentors have insights and information that can be beneficial to both patients and their caregivers.

Contact the Patient Navigation Center at 888-841-6673 to be connected to a Myeloma Mentor or to learn more.

Join the MMRF Community!

National Walk/Run Program

- Atlanta | 10.26.24
- Boston | 10.12.24
- Chicago | 9.8.24
- Dallas | 11.16.24
- Detroit | 9.21.24
- Houston | 11.23.24
- Los Angeles | 8.17.24
- National Virtual | 12.14.24
- New York City | 10.5.24
- Philadelphia | 10.19.24
- San Francisco | 8.24.24
- Scottsdale | 12.7.24
- Tampa | 11.2.24
- Twin Cities | 9.14.24
- Washington D.C. | 9.28.24



Other MMRF Event Programs



Moving Mountains for Multiple Myeloma



Half and Full Marathons



Bike/Road to Victories



Create Your Own Fundraiser



Upcoming Patient Education Events

Save the Date

Program	Date and Time	Speakers
Patient Summit New York, NY & Virtual <i>(In collaboration with Mount Sinai)</i>	Saturday, May 4, 2024 9:00 AM – 12:30 PM ET 6:00 AM – 9:30 AM PT	Hearn Jay Cho, MD, PhD Leora Giacoia, NP, FNP-BC Sundar Jagannath, MD Joshua Richter, MD Samir Parekh, MD Shambavi Richard, MD Cesar Rodriguez, MD Santiago Thibaud, MD
Patient Webinar <i>Understanding Your Lab Report</i>	Monday, May 13, 2024 3:30 PM – 4:30 PM ET 12:30 PM – 1:30 PM PT	Craig E. Cole, MD Amy Blake, NP-C

For more information or to register, visit themmr.org/educational-resources

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Resources

- Resource tab includes
 - Exhibit Hall
 - Speaker bios
 - Copy of the slide presentation

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Need help with travel to a clinical study?

- The MMRF has partnered with the Lazarex Cancer Foundation to help provide more equitable access to clinical studies for multiple myeloma patients
- This partnership is one facet of the MMRF's commitment to improve diversity and representation in myeloma clinical studies
- MMRF has provided \$100,000 over 2 years to Lazarex to fund travel, lodging, and food for patients (and a travel companion) so that they can participate in clinical studies that are appropriate for them
- Patients are funded according to income guidelines and will be reimbursed for allowed expenses
- For more information on this program and to be connected with Lazarex, call our Patient Navigation Center at 1-888-841-6673



Thank you!