MULTIPLE MYELOMA
Research Foundation

MULTIPLE MYELOMA **AUTOLOGOUS STEM CELL TRANSPLANTATION**

themmrf.org

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ABOUT THE **MMRF**

The Multiple Myeloma Research Foundation® (MMRF®) is the largest nonprofit in the world solely focused on accelerating a cure for each and every multiple myeloma patient. We drive the development and delivery of next-generation therapies, leverage data to identify optimal and more personalized treatment approaches, and empower myeloma patients and the broader community with information and resources to extend their lives.

Central to our mission is our commitment to advancing access for all so that every myeloma patient can benefit from the scientific and clinical advances we pursue. Since our inception, the MMRF has raised over $600 million for research, opened over 100 clinical trials, and helped bring more than 15 FDA-approved therapies to market, which have tripled the life expectancy of myeloma patients.

To learn more about the MMRF, visit **themmrf.org**.

To speak to a patient navigator at the Patient Navigation Center, call **1-888-841-6673** or email **patientnavigator@themmrf.org**.

Accredited by:

INTRODUCTION

For newly diagnosed **multiple myeloma** patients who are generally in good health and have the necessary support, **autologous stem cell transplant (ASCT)** offers one of the best chances for a deep, long-lasting **remission**. According to the National Comprehensive Cancer Network Clinical Practice Guidelines, ASCT remains the standard of care for eligible newly diagnosed patients.

This booklet is designed to help you better understand ASCT: what it is, what it involves, and how it helps. Words that may be unfamiliar are **bolded** and defined in the Glossary (page 10).

For an overview of ASCT, see the ***ASCT* High-Impact Topic** video.

bit.ly/ASCT_HIT

The information in this booklet is not intended to replace the services or advice of trained health care professionals. Please consult with your care provider regarding specific questions relating to your health, especially questions about myeloma diagnosis or treatment.

For more information about multiple myeloma and its treatment, refer to the other booklets in our Patient Toolkit, as well as the MMRF website, **themmrf.org**.

WHAT IS ASCT?

In ASCT, your blood **stem cells** are collected and then transplanted back into you after you receive high-dose **chemotherapy**.

Blood stem cells are immature cells that can develop into any type of blood cell. They're normally found in the **bone marrow** and in the peripheral blood (blood in the arteries or veins).

Is ASCT right for you?

Guidelines for patient suitability for ASCT may vary between cancer centers. Discuss your suitability, as well as the risks and benefits, with a myeloma specialist. If chemotherapy and ASCT is an option, your myeloma specialist can also determine when it should be included in your treatment plan.

If you aren't eligible for ASCT, there are still many effective treatments available. These include combinations of drugs that control myeloma and reduce symptoms.

If you forego ASCT, you can still have successful outcomes with other treatments. Your doctor will choose and adjust treatments to fit your needs. Newer treatments continue to improve how long and how well people live with myeloma, even without ASCT.

If you're a candidate for transplant but aren't sure whether you want to undergo the procedure, it's still recommended to have stem cells collected so they're available if you decide to undergo ASCT in the future. The cells can be frozen, stored for decades, and used later.

TOLERATING HIGH-DOSE CHEMOTHERAPY AND ASCT

Today, more people are able to receive high-dose chemotherapy and ASCT than in the past. This is because doctors now look at overall health—not just age—when deciding if this procedure is right.

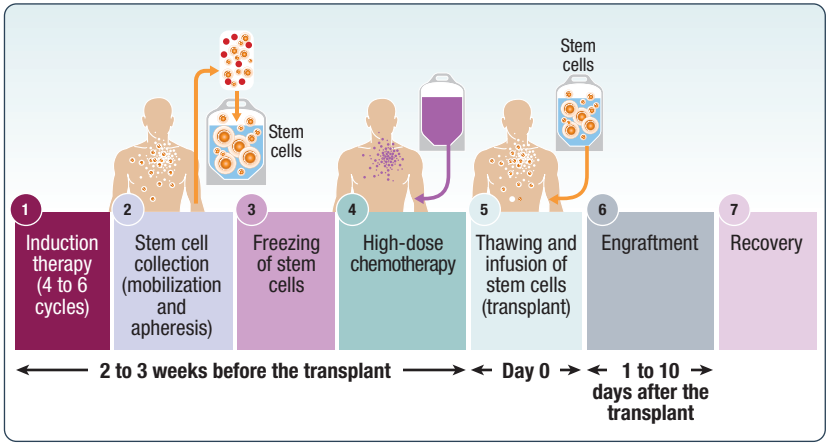
When considering you for an ASCT, your care team will look at factors such as:

- Fitness: your general activity level and how active you are day to day
- Frailty: how many other health conditions (**comorbidities**) you have
- Ability to tolerate treatment: how well your body can handle high-dose chemotherapy

HIGH-DOSE CHEMOTHERAPY AND ASCT: THE PROCESS

The process of getting high-dose chemotherapy and ASCT involves several steps.

The ASCT process.



INDUCTION THERAPY

In the weeks leading up to your chemotherapy and transplant, you'll receive at least four cycles of **induction therapy** (typically a combination of four medications, including a **proteasome inhibitor**, an **immunomodulatory drug**, a **monoclonal antibody**, and a steroid) to ensure that the number of myeloma cells in your body is as low as possible.

STEM CELL COLLECTION

An important part of stem cell collection is encouraging your body to increase its production and release of healthy stem cells into your bloodstream; these are the stem cells that will be collected and transplanted back into you.

Mobilization

Following induction therapy, you'll be given drugs called **colony stimulating factors** to help your body produce more stem cells. This process is called **mobilization**.

Apheresis

The next step is **apheresis**, which involves a machine that separates stem cells from your blood and returns the rest of your blood to you. This procedure is usually done in daily visits to a clinic (outpatient) and takes about 3 to 4 hours a day over 1 to 5 days.

What to expect during apheresis:

- Bruising
- Muscle cramps
- Twitching
- Tingling sensation in your fingertips or lips
- Nausea/diarrhea
- Bone pain
- Headache
- Flu-like symptoms

Enough stem cells will be collected so that at least two rounds of ASCT can be performed, if needed. Collected stem cells are frozen and stored until you're ready for them to be re-infused.

HIGH-DOSE CHEMOTHERAPY AND STEM CELL INFUSION

Chemotherapy kills myeloma cells, but it also destroys normal blood-forming cells in your bone marrow and reduces the effectiveness of your **immune system**.

The amount of time between when your stem cells are collected and re-infused can vary from days to years, depending on when you choose to undergo the procedure. Deciding when to have high-dose chemotherapy is what will determine the timing of your stem cell transplant.

The stem cell infusion replaces the cells that were killed along with the myeloma cells by the chemotherapy.

What to expect:

- You'll receive an infusion of high-dose melphalan, a strong chemotherapy drug that kills most myeloma cells left in your body
- 1 to 2 days after receiving melphalan, your previously collected stem cells are returned to you through an infusion (the actual transplant)
- The entire process usually takes about 2 to 3 weeks

You can undergo stem cell infusion as an inpatient (you stay in the hospital before, during, and immediately after the transplant) or an outpatient procedure.

ENGRAFTMENT

Within 2 weeks of the transplant, newly formed blood cells can be detected in your blood—a process called **engraftment**. Your blood counts will steadily increase over time.

A successful transplant results in the growth of healthy **red blood cells, white blood cells**, and **platelets**.

SIDE EFFECTS

During and after your transplant, you may experience side effects, including fatigue, nausea and vomiting, diarrhea, and mouth sores (mucositis). One of the biggest risks is low blood counts, which increase your risk of infection.

Side effects and management.

Side effect	Description	Management
Low blood counts	• White blood cells drop to zero, raising infection risk • Hemoglobin and platelets drop • Counts begin to recover 10–12 days after chemotherapy	• **Prophylactic antimicrobials** • Transfusion with blood/platelets
Nausea and vomiting		• Symptoms much more manageable with newer drugs
Diarrhea	• May include stomach cramping	• Eat small amounts of food, more often • Avoid milk, milk products, high-fiber foods
Mucositis	• Pain • Sores in mouth • Sore throat	• Avoid tart, acidic, salty, spicy foods • Soft food better tolerated • Chewing on ice
Fatigue	• Common • May last 1–3 months	• Rest • Good nutrition • Exercise
Hair loss	• Temporary	• Gentle hair care • Wigs, scarves, hats
Organ damage (lungs, liver kidneys)	• Rare	• Care team will follow closely with testing • Adjust medicines, fluids, or doses to protect these organs and treat any problems early.

While you are undergoing ASCT, you'll be carefully monitored, and **supportive care** will be provided to minimize and manage side effects.

Infection prevention.

AFTER ASCT

The transplant replaces the stem cells that were killed along with the myeloma cells by the chemotherapy. However, it takes time for these cells to grow, so you'll be monitored closely.

After your transplant, you'll need a caregiver for several weeks. Caregivers can help you in many ways:

- Taking you to and from appointments
- Making sure you take your medications
- Keeping an eye out for changes in your condition
- Advocating for your needs
- Helping with decision-making
- Preparing meals
- Helping you bathe
- Providing emotional support

Starting about 60 to 100 days after transplant, you'll receive several follow-up tests:

- Blood tests
- Imaging
- **Bone marrow biopsy**
- Possibly, measurement of **minimal residual disease**, which can determine the number of myeloma cells that remain after ASCT

Treatment After ASCT

After your transplant, your doctor will recommend a plan for **consolidation therapy** and/or **maintenance therapy**. This plan will be based on whether you have standard- or high-risk myeloma.

Post ASCT treatment of standard- and high-risk myeloma.

Risk	Treatment
Standard-risk	Maintenance therapy with Revlimid (possibly with other drugs like Velcade or Darzalex) begins 100 days after the transplant. This timing is best for introducing a new treatment, because blood cell counts will be nearly back to normal
High-risk	Maintenance therapy may begin 60 days after the transplant, and additional drugs may be given with Revlimid. The reason for the shorter time frame is that, for high-risk myeloma, maintaining disease control is the highest priority

In the U.S., maintenance is typically given until your myeloma progresses. However, clinical trials are looking at stopping maintenance treatment for patients with deep responses.

If you have little or no evidence of disease but are experiencing side effects, discuss options for continuing maintenance treatment with your doctor.

Following ASCT, it may take several months for you to be able to resume normal activities.

You may need to schedule time off from work. Depending on your job's physical demands and whether it carries a higher risk for infection, you may need to work part-time when you return and then ramp up to a full-time schedule.

Taking care of your overall health in the months after your ASCT will give you the best chance for a faster, fuller recovery.

The road to recovery.

THE EVOLVING VIEW OF ASCT IN MYELOMA

The improved response rates seen in initial therapy with today's myeloma regimens have raised questions about the timing of ASCT for myeloma.

Studies have found that doing an ASCT early for newly diagnosed myeloma leads to a longer period before the disease comes back but does not increase how long patients live compared to using drugs alone and saving transplant for later.

These results suggest that modern treatments give patients and doctors more flexibility to tailor therapy, allowing some patients to safely delay or avoid early transplant. The timing of ASCT remains an area of ongoing investigation.

For now, however, induction therapy followed by high-dose chemotherapy and ASCT remains a standard therapy, and this option may offer the best chance for a long-lasting remission for suitable candidates.

Questions to ask your doctor about ASCT.

- Am I a candidate for high-dose chemotherapy and stem cell transplantation?
- When is the best time for me to undergo ASCT?
- Does your center do ASCT?
- How many transplants has your center performed in multiple myeloma in the last year?
- Does your center perform ASCT on an inpatient or outpatient basis?
- 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 receive more than one transplant?

ASCT RESOURCES

Blood & Marrow Transplant Information Network
Visit **bmtinfonet.org**

National Bone Marrow Transplant Link
Visit **nbmtlink.org**

Bone Marrow & Cancer Foundation
Visit **bonemarrow.org**

The MMRF would like to thank Joshua Richter, MD, Associate Professor of Medicine, Hematology and Oncology, in the Myeloma Division at the Tisch Cancer Institute at the Icahn School of Medicine at Mount Sinai and Director of Myeloma at the Blavatnik Family Chelsea Medical Center at Mount Sinai and our patient advocate, Kerri Hoffman of Carlsbad, California, for their contributions to this booklet.

GLOSSARY

antibody Protein produced by *plasma cells* that helps protect the body from infection and disease

antimicrobials Drugs that kill or slow the growth of bacteria, viruses, fungi, and parasites

apheresis Procedure in which blood is collected from a patient, part of the blood (such as white blood cells) is taken out, and the rest of the blood is returned to the patient

autologous stem cell transplant (ASCT) Procedure in which stem cells collected from a patient are transplanted back into that patient; the most common type of transplant performed in myeloma

bone marrow Soft, spongy tissue found in the center of many bones and the site of blood cell production

bone marrow biopsy Removal of a sample of bone marrow for examination; performed using a needle

chemotherapy Use of drugs to kill rapidly dividing cells, such as cancer cells

colony-stimulating factors *Growth factors* that stimulate the bone marrow to produce white blood cells

comorbidities Diseases that are present at the same time as another disease

consolidation therapy Short-term treatment given to a patient after initial treatment to target remaining cancer cells

engraftment Process by which stem cells that have been infused into the body start to grow and make new blood cells

growth factors Substances that stimulate cells to multiply

immune system Network of cells that protect the body from foreign substances and can destroy infected and cancerous cells

immunomodulatory drug Drug that fights cancer by boosting the immune system; examples include Thalomid, Revlimid, and Pomalyst

induction therapy The first treatment a patient receives for myeloma after he or she is diagnosed; also refers to the use of anti-myeloma drugs prior to high-dose chemotherapy and stem cell transplant (also known as frontline therapy)

maintenance therapy Treatment given over a long period of time to patients in remission to reduce the risk of *relapse*

minimal residual disease Presence of small numbers of myeloma cells in the bone marrow during or after treatment, even when the patient shows no symptoms or signs of disease

mobilization Process of stimulating stem cell growth to ensure that enough stem cells can be collected for transplantation

monoclonal antibody Antibody produced in a lab that is used to diagnose and treat some diseases

multiple myeloma Blood cancer that develops in the bone marrow as a result of *plasma cells* transforming into myeloma cells

plasma cells *Antibody*-secreting immune cells that develop from B cells; in myeloma, it is these cells that have become cancerous or abnormal

platelets Small cell fragments in the blood that help it to clot

prophylactic Preventing the spread or occurrence of infection or disease

red blood cells Blood cells that carry oxygen

relapse Progression of a disease that initially responded to therapy

remission Period when the signs and symptoms of a disease lessen or disappear

stem cells Cells that grow and divide to produce red blood cells, white blood cells, or platelets; found in bone marrow and blood

supportive care Treatment that addresses the symptoms and complications of a disease rather than the disease itself

white blood cells One of the major cell types in the blood; attack infection and cancer cells as part of the immune system

NOTES

MMRF PATIENT SUPPORT AND RESOURCES

The MMRF supports the myeloma community by providing a broad range of resources for myeloma patients and their family members and caregivers. The MMRF is available to help guide you through your multiple myeloma journey every step of the way.

YOUR QUESTIONS ANSWERED

The Patient Navigation Center is available to answer your questions about disease management and treatments, help you find clinical trials, and connect you with financial and other resources.

Telephone: 1-888-841-6673
Monday—Friday, 9:00 AM to 7:00 PM ET
Email: **patientnavigator@themmrf.org**

themmrf.org/support/patient-navigation-center

CONNECT WITH AN MMRF MYELOMA MENTOR

Connect one-on-one with a trained patient and/or caregiver mentor that can share their patient journeys and experiences.

themmrf.org/support/myeloma-mentors

FIND A CLINICAL TRIAL

The MMRF Clinical Trial Finder lets you search for a clinical trial in your area.

themmrf.org/diagnosis-and-treatment/clinical-trials-and-emerging-therapies/clinical-trial-finder/

VIEW PAST PROGRAMS ON DEMAND

Access our archive of recorded Patient Summits and webcasts. Hear expert perspectives on key clinical research and the rapidly evolving myeloma treatment landscape. All available online, and free.

themmrf.org/educational-resources

SUPPORT THE MMRF

Help support the MMRF's efforts to accelerate research and find a cure! Participate in an event or donate today.

Telephone: 1-203-229-0464
Donate now/Take action: **themmrf.org/get-involved**

ATTEND A MULTIPLE MYELOMA PATIENT SUMMIT

Available in-person and virtually, MMRF Patient Summits discuss new treatments, promising clinical trials, and all the information you need to make well-informed decisions about your treatment and care.

themmrf.org/educational-resources

Don’t miss out on the latest myeloma updates!
Sign up today to receive news updates and notice of educational programs.

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I AM A:

- ☐ Myeloma Patient
- ☐ Myeloma Patient Caregiver
- ☐ Myeloma Patient Family Member (non-caregiver)
- ☐ Family/Friend of Deceased Myeloma Patient
- ☐ Healthcare Professional or Researcher
- ☐ Biopharma, Medical Device, or Healthcare Technology Industry Professional
- ☐ None of the Above

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Hours: **Mon–Fri, 9 AM–7 PM ET**
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