



MULTIPLE MYELOMA
Research Foundation

MULTIPLE MYELOMA AUTOLOGOUS STEM CELL TRANSPLANTATION

themmrf.org





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 health equity so that all myeloma patients can benefit from the scientific and clinical advances we pursue. Since our inception, the MMRF has committed over \$500 million for research, opened nearly 100 clinical trials, and helped bring 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.

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INTRODUCTION

For newly diagnosed **multiple myeloma** patients who are eligible, an **autologous stem cell transplant (ASCT)** offers the best chance for a deep, long-lasting remission. According to the National Comprehensive Cancer Network Clinical Practice Guidelines, ASCT remains the standard of care for newly diagnosed, eligible 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 11).

For more information about multiple myeloma and its treatment, refer to the companion booklets ***Multiple Myeloma Disease Overview***, ***Newly Diagnosed Multiple Myeloma***, ***Multiple Myeloma Treatment Overview***, and ***Multiple Myeloma Immunotherapy*** and the MMRF website, **themmrf.org**.

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

HIGH-DOSE CHEMOTHERAPY AND THE ASCT PROCEDURE

High-dose **chemotherapy** with ASCT (if you are eligible) offers the best chance for long-lasting remission. Chemotherapy is effective in killing myeloma cells, but it also destroys normal blood-forming cells in your **bone marrow** and reduces the effectiveness of your **immune system**. ASCT uses your blood **stem cells**—cells that can develop into many different types of blood cells, also called **peripheral blood stem cells (PBSCs)**—to restore these important cells and help your body recover from the chemotherapy.

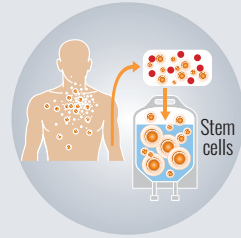
PBSC COLLECTION AND INFUSION

PBSCs are normally found in the bone marrow and in the peripheral blood (blood in the arteries or veins). Almost all transplants in myeloma are now performed using stem cells from a patient's own (autologous) peripheral blood and are referred to as ASCT or autologous PBSC transplants. Bone marrow transplants are rarely done in multiple myeloma.

In the weeks leading up to your transplant, stem cells are collected (**Box**)—a process known as **apheresis**—after you receive at least three cycles of **induction therapy** to ensure that the number of myeloma cells in your body is as low as possible.

Stem cell collection (apheresis)

During apheresis, you are connected to a machine that separates the PBSCs from your blood and returns the blood to you. This procedure typically takes 3 to 4 hours over the course of 1 to 5 days and can be performed on an outpatient basis.



During this procedure, you may experience bruising at the site of needle insertion or muscle cramps, twitching, or a tingling sensation in your fingertips or lips.

In the days leading up to your apheresis, you will receive drugs to make sure that enough PBSCs can be collected for several transplants, if needed. These drugs include **colony-stimulating factors** (for example, Neupogen, Neulasta, and Leukine) and a drug called Mozobil (plerixafor). This process of stimulating the growth of PBSCs is known as **mobilization**.

Apheresis is tolerable for most patients; common side effects of some of the drugs used for mobilization include bone pain, headaches, flu-like symptoms, or nausea and diarrhea.

Once the PBSCs are collected, they are frozen and stored until you are ready for them to be re-infused. You will receive an infusion of high-dose melphalan, a strong chemotherapy drug that kills most myeloma cells left in your body. At least 24 hours later, usually 1 to 2 days after receiving melphalan, the PBSCs are thawed and returned to you through an infusion.

The amount of time between when your PBSCs are collected and re-infused depends on the timing of your transplant and can vary from days to years, depending on when you choose to undergo the ASCT. Your PBSCs can be stored for decades.

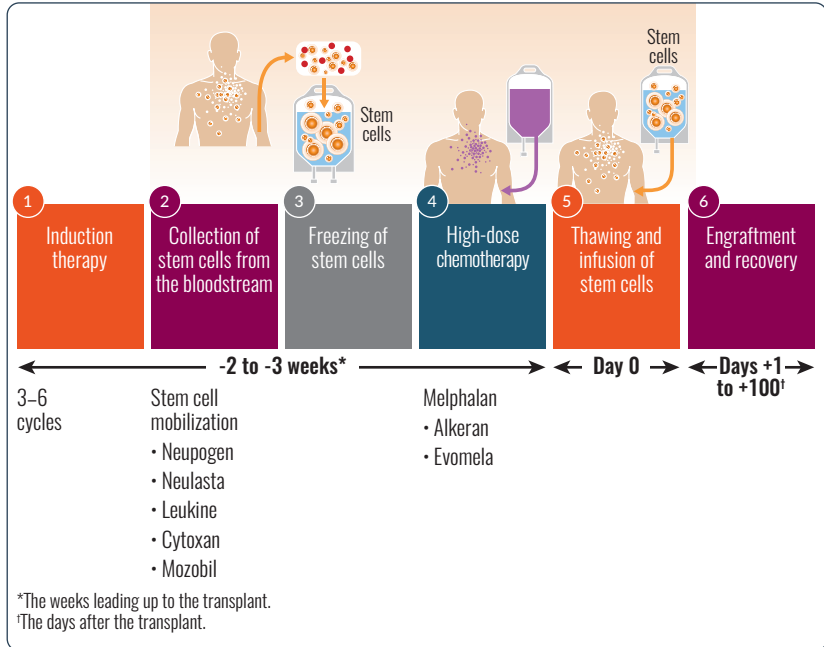
Unless you choose to wait to undergo ASCT, the process—from melphalan chemotherapy to PBSC re-infusion and initial recovery—usually takes about 3 weeks.

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. Typically, you can expect to be in the hospital or visit an outpatient clinic for an average of 3 weeks for treatment and recovery.

ASCT ENGRAFTMENT, RECOVERY, AND SIDE EFFECTS

Within 2 weeks of the transplant, newly formed blood cells can be detected in your blood. Your blood counts will steadily increase over time—a process called **engraftment**. A successful transplant results in the growth of healthy **red blood cells**, **white blood cells**, and **platelets**.

The ASCT process.



You may experience side effects, including fatigue, nausea and vomiting, diarrhea, mouth sores (mucositis), and low blood counts.

High-dose chemotherapy: side effects and management.

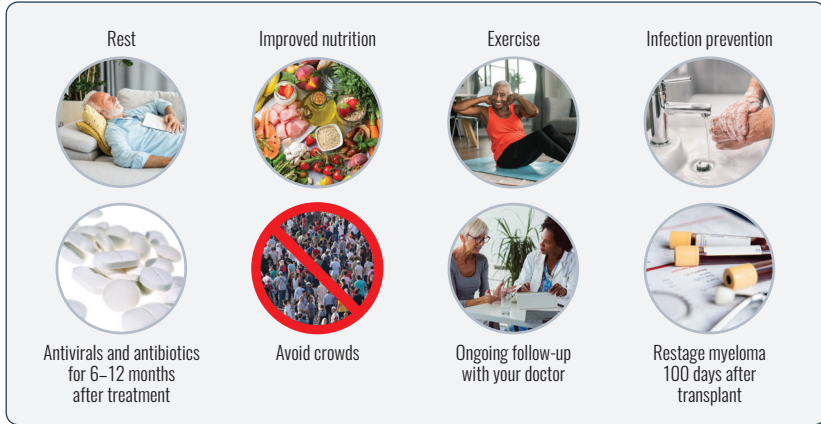
| Side Effect | Description | Management |
|---------------------|--|--|
| Fatigue | <ul style="list-style-type: none"> • Expected • May last 1–3 months | <ul style="list-style-type: none"> • Rest • Practice good nutrition • Exercise |
| Nausea and vomiting | | <ul style="list-style-type: none"> • Symptoms much more manageable with newer antiemetics |
| Diarrhea | <ul style="list-style-type: none"> • May include stomach cramping | <ul style="list-style-type: none"> • Eat small amounts of food, more often • Avoid milk, milk products, high-fiber foods |
| Mucositis | <ul style="list-style-type: none"> • Pain, sores in mouth, sore throat | <ul style="list-style-type: none"> • Avoid tart, acidic, salty, spicy foods • Soft food better tolerated |
| Low blood counts | <ul style="list-style-type: none"> • White blood cells drop to zero, raising infection risk • Hemoglobin and platelets drop • Counts begin to recover 10–12 days after chemotherapy | <ul style="list-style-type: none"> • Prophylactic antimicrobials • Transfusion with blood/platelets |

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

Because high-dose chemotherapy attacks healthy disease-fighting cells as well as cancerous cells, you will be at an increased risk of infection. It is important that you follow all of your health care team’s directions to minimize the risk of infection. Hair loss is also a common side effect. Other possible but uncommon side effects include damage to the lungs, liver, and kidneys. Additionally, you may experience fatigue, which can last 1–3 months. It may take several months for you to be able to resume normal activities.

Ongoing monitoring may include blood tests, imaging, **bone marrow biopsies**, and measurement of **minimal (measurable) residual disease (MRD)**, which can determine the number of myeloma cells that remain after ASCT.

The road to recovery.



After ASCT, your doctor will recommend a personalized plan for **consolidation therapy** and/or **maintenance therapy**.

CANDIDATES FOR ASCT

More patients are considered to be candidates for ASCT today than in the past because age is no longer considered a barrier to transplant. Rather, a patient's suitability for high-dose chemotherapy and transplant is based on overall health.

A variety of factors such as fitness and frailty influence whether you are eligible for ASCT. For example, the presence of multiple **comorbidities** and your activity level can affect your response to, adherence to, and tolerance of treatments, including ASCT. It is important that treatment decisions for you consider not just your age but also your clinical and functional status.

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

If you are eligible for transplant, you are encouraged to have stem cells collected so that the cells are available if you choose to undergo the process at some point during the course of your disease.

THE EVOLVING VIEW OF TIMING FOR ASCT IN MYELOMA

The improved response rates seen in initial therapy with today's myeloma regimens have raised questions about the timing of ASCT in myeloma treatment. A European and US study compared early ASCT (that is, ASCT performed right after induction, shortly after diagnosis) to late ASCT (performed after **relapse**) and showed that patients who got an early transplant tended to have a longer time without relapse than did those who got a late transplant. This result does not mean that all patients necessarily live longer after receiving an early transplant; however, those who did receive an early transplant were able to maintain their low disease status without progressing for a longer time than were patients who received a late transplant. For now, early ASCT remains a standard therapy and may offer the best chance for a long-lasting remission for suitable candidates.

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?



ASCT RESOURCES

Blood & Marrow Transplant Information Network

Visit www.bmtinfonet.org

National Bone Marrow Transplant

Visit www.nbmtlink.org

BMT Support Online

Visit www.bmtsupport.org

Bone Marrow and Cancer Foundation

Visit www.bonemarrow.org

The MMRF would like to thank Amrita Y. Krishnan, MD, Professor, Department of Hematology & Hematopoietic Cell Transplantation, Director, Judy and Bernard Briskin Center for Multiple Myeloma Research at the City of Hope Medical Center in Duarte, California, and our patient advocate Colin Todd Kennedy of Coto de Caza, California, for their contributions to this booklet .

MMRF PATIENT SUPPORT AND RESOURCES

The MMRF is dedicated to supporting 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

Speak to an MMRF patient navigator at the Patient Navigation Center for answers to your questions about disease management, treatments, clinical trials, and assistance with finding financial and other available resources.

Telephone: 1-888-841-6673

Monday–Friday, 9:00 AM to 7:00 PM ET

Email: patientnavigator@themmrf.org

Online: themmrf.org/support/patient-navigation-center

Connect with an MMRF Myeloma Mentor™:

themmrf.org/support/myeloma-mentors

This is a phone-based program offering the opportunity for patients and/or caregivers to connect one-on-one with a trained patient and/or caregiver mentor to share their patient journeys and experiences.

FIND AND PARTICIPATE IN A CLINICAL TRIAL

Search for a clinical trial in your area or let an MMRF patient navigator help guide you through the process.

Clinical Trial Finder: themmrf.org/resources/clinical-trial-finder

The MMRF has partnered with Lazarex Cancer Foundation to help patients access clinical trials by helping with travel expenses. Patients who qualify will be reimbursed for out-of-pocket travel expenses for themselves and a travel companion. To learn more about this program, contact the MMRF Patient Navigation Center (1-888-841-6673 or patientnavigator@themmrf.org).

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: Visit themmrf.org/get-involved

GLOSSARY

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

antiemetic Drug that prevents or relieves nausea and vomiting

antimicrobial Drug that kills or slows 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 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 cancer cells

colony-stimulating factor *Growth factor* that stimulates the bone marrow to produce white blood cells

comorbidity Medical condition that is present at the same time as another condition

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

frontline therapy The first multiple myeloma treatments received (see also *induction therapy*)

growth factor Substance that stimulates cells to multiply

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

induction therapy The first treatment a patient receives for myeloma; also refers to the use of anti-myeloma drugs prior to high-dose chemotherapy and stem cell transplant (see also *frontline therapy*)

maintenance therapy Treatment that is given to patients following a response to induction therapy over a long period of time to reduce the risk of relapse

minimal (measurable) residual disease (MRD) 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

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

peripheral blood stem cell (PBSC) Stem cell collected from the blood

plasma cell *Antibody*-secreting immune cell that develops from a B cell; in myeloma, it is this cell that has become cancerous

platelet Small cell fragment in the blood that helps it to clot

prophylactic Preventing the spread or occurrence of disease

red blood cell Blood cell that carries oxygen

relapse Progression of myeloma after an initial response to therapy

stem cell Cell that grows and divides to produce red blood cells, white blood cells, and platelets; found in bone marrow and blood

supportive care Physical, psychological, social, and spiritual support for patients and their caregivers

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



MMRF RESOURCES IN PERSON OR ONLINE



Attend a Multiple Myeloma Patient Summit

Learn about standard and emerging therapies, including stem cell transplants, promising clinical trials, and more for optimal disease management. Attend a complimentary symposium for all the information you need to make well-informed decisions about your treatment and care.

To register or to view the complete calendar, visit:
themmrf.org/resources/education-programs



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, at:
themmrf.org/resources/education-programs



Find a Clinical Trial Near You

Clinical trials are critically important to developing new myeloma treatments and better understanding the biology of the disease. The more people who enroll, the faster we can find answers. Patients who enroll in clinical trials have the opportunity to be among the first to receive the newest drugs or drug combinations in development and receive close monitoring.

To find a clinical trial near you, visit:
themmrf.org/resources/clinical-trial-finder

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

Name: _____

Address: _____

City: _____

State: _____

ZIP: _____

Telephone: _____

Mobile: _____

Email: _____

Or sign up at **themmrf.org**

I AM A:

- Myeloma Patient
- Myeloma Patient Caregiver
- Myeloma Patient Family Member (non-caregiver)
- Health Care Professional or Researcher
- Biopharma, Medical Device, or Health Care Technology Industry Professional

**Please tear off reply card and tape all three sides before mailing.*

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