



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 raised over \$600 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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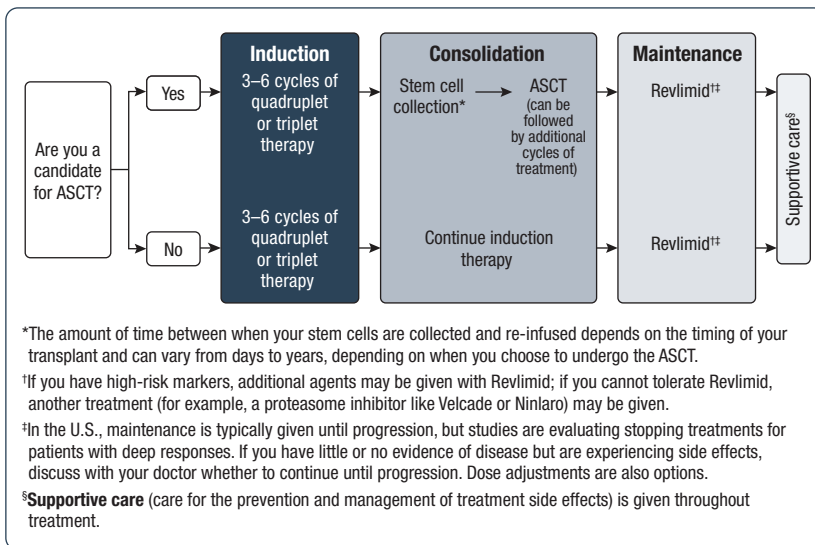
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INTRODUCTION

For newly diagnosed **multiple myeloma** patients who are generally in good health and have the necessary support, 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.

Treatment approach for 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 11).

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

For many patients, an initial treatment that includes high-dose **chemotherapy** and ASCT 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** to restore these important cells and help your body recover from the chemotherapy. Stem cells are cells that can develop into many different types of blood cells.

CANDIDATES FOR ASCT

More patients are considered to be candidates for ASCT today than in the past. This is because a patient's suitability for high-dose chemotherapy and transplant is now based on overall health rather than age alone.

Factors such as fitness (for example, your activity level) and frailty (for example, how many **comorbidities** you have) influence whether you are able to undergo ASCT. These are factors that can affect your ability to stick with and tolerate treatment, as well as how you will respond to the treatment. It's important that treatment decisions are made with your general health, including your organ function and your ability to perform daily activities, in mind.

Guidelines for patient suitability for ASCT may vary between cancer centers. You should discuss your suitability 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 a candidate 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. It is possible for cells to be frozen and used years later, and centers will often collect enough stem cells for more than one transplant in the event that a second transplant is needed. In addition, cells can be shipped to another treatment center if you move or change doctors.

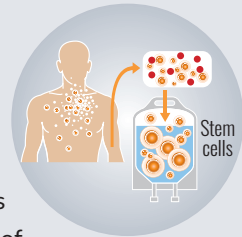
STEM CELL COLLECTION

Blood stem cells are immature cells that can develop into any type of blood cell and 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. Bone marrow transplants are rarely done in multiple myeloma.

In the weeks leading up to your transplant, you will receive at least three cycles of **induction therapy** (typically a combination of three to four medications) to ensure that the number of myeloma cells in your body is as low as possible. After that, stem cells are collected in a process called **apheresis**. Once the stem cells are collected, they are frozen and stored until you are ready for them to be re-infused.

Stem cell collection (apheresis)

During apheresis, you are connected to a machine that separates the stem cells 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 stem cells are collected for at least two transplants, if needed. These drugs include **colony-stimulating factors** (for example, Neupogen, Neulasta, and Leukine) and Mozobil or Apherda. This process of stimulating the growth of stem cells is known as **mobilization**. Some patients may also require a dose of chemotherapy to help increase the number of stem cells that can be collected.

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

STEM CELL INFUSION

The amount of time between when your stem cells 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 stem cells can be stored for decades. When it's time for your transplant, you'll 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 stem cells are thawed and returned to you through an infusion.

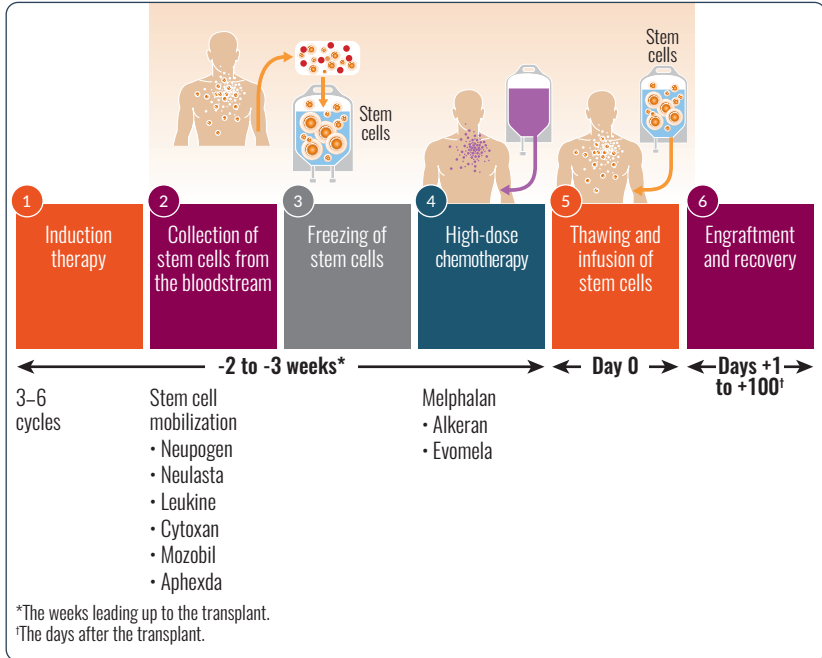
Unless you choose to wait to undergo ASCT, the entire process usually takes about 2–3 weeks.

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 2–3 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
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
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	Pain, sores in mouth, sore throat	<ul style="list-style-type: none">• Avoid tart, acidic, salty, spicy foods• Soft food better tolerated• Chewing on ice
Fatigue	<ul style="list-style-type: none">• Expected• May last 1–3 months	<ul style="list-style-type: none">• Rest• Good nutrition• Exercise

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

AFTER ASCT

After your transplant, you'll need a caregiver to help you manage your medical care and household tasks for several weeks or months. A caregiver is someone who supports you in a number of ways during and after treatment. This includes 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 food, helping you bathe, and providing emotional support.

Starting about 60–100 days after transplant, you will receive several follow-up tests. Ongoing monitoring may include blood tests, imaging, **bone marrow biopsies**, and potential measurement of **minimal (measurable) residual disease (MRD)**, which can determine the number of myeloma cells that remain after ASCT.

You will continue treatment after your transplant. Your doctor will recommend a personalized plan for **consolidation therapy** and/or **maintenance therapy**. For patients with standard-risk myeloma, maintenance therapy with Revlimid (possibly along with other medications like Velcade or Darzalex) begins 100 days after the transplant. This timing is best for introducing a new treatment, because your blood cell counts will be nearly back to normal.

For patients with high-risk myeloma (those at highest risk for relapse), maintenance therapy may begin 60 days after the transplant and additional agents may be given with Revlimid. The reason for the shorter time frame is that maintaining disease control is the highest priority for these patients.

In the U.S., maintenance is typically given until your myeloma progresses, but 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 with your doctor whether to continue your maintenance treatment.

High-dose chemotherapy attacks healthy disease-fighting cells as well as cancerous cells. Because of this, you'll be at an increased risk of infection. It's important that you follow all of your health care team's directions to minimize the risk of infection. This may include practicing good personal hygiene, frequent hand washing, and avoiding crowds and sick people when possible. You or your caregiver should report any fever of more than 100.4°F to your health care team. Starting 6 months after your transplant, you should also receive all of your vaccines again.

Hair loss is a common side effect of the chemotherapy used to kill myeloma cells, but it's temporary. Other possible but uncommon side effects include damage to the lungs, liver, and kidneys. You may feel tired and weak, especially in the days right after the transplant, but this can last 1 to 3 months. It may take several months for you to be able to resume normal activities. The length of time you will have to take off work after ASCT is about 3 to 6 months. This time may vary 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 first return and then slowly ramp up to a full-time schedule.

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 in myeloma treatment. A European study compared early ASCT (that is, ASCT performed right after induction, shortly after diagnosis) to late ASCT (performed after **relapse**) and showed that the time to remission was longer in patients who got an early transplant than in those who got a late transplant. A US study comparing patients treated with Revlimid, Velcade, and dexamethasone (RVd) plus early ASCT to patients treated with RVd alone (no transplant) showed similar results. These results do not mean that all patients necessarily live longer after receiving an early transplant. They do show, however, that patients who received 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 or no transplant. For now, early 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 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 Link

Visit www.nbmtlink.org

BMT Support Online

Visit www.bmtsupport.org

Bone Marrow & Cancer Foundation

Visit www.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, Andrew Gordon of Harrisburg, Pennsylvania, 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

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 (also called *immunoglobulin*; see also *monoclonal antibody*)

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 First treatment given to a newly diagnosed patient (also known as *induction therapy*, first-line therapy, or frontline treatment)

growth factor Substance that stimulates cells to multiply

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

immunoglobulin (Ig) Protein that helps protect the body from infection (also called *antibody*)

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 (see also *frontline therapy*)

maintenance therapy Treatment given over a long period of time to patients in remission 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

monoclonal antibody Antibody produced in a laboratory 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 cell *Antibody*-secreting immune cell that develops from a B cell; in myeloma, it is this cell that has 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 cell Blood cell that carries oxygen

relapse Progression of a disease after an initial response to therapy

stem cell Cell that grows and divides 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; examples in myeloma include bisphosphonates, growth factors, antibiotics, orthopedic interventions, and pain control measures

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.

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or Researcher |
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Care Technology Industry Professional |
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