MULTIPLE MYELOMA
Research Foundation

MULTIPLE MYELOMA TREATMENT OVERVIEW

themmrf.org

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Research Foundation

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**.

Accredited by:

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INTRODUCTION

Patients with **multiple myeloma** have more treatment options than ever before. Over the last two decades, more than 15 treatments have been approved by the US Food and Drug Administration (FDA) for the treatment of myeloma, and many other therapies are under investigation.

This booklet is designed to help you better understand the current treatment options for multiple myeloma, as well as the emerging treatment options that are being tested in clinical trials. Words that may be unfamiliar are **bolded** and defined in the Glossary (page 37).

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 provider regarding specific questions relating to your health, especially questions about myeloma diagnosis or treatment.

For more information about how myeloma develops, as well as its symptoms, diagnosis, and **prognosis**, refer to the companion booklet ***Multiple Myeloma Disease Overview*** and the MMRF website, **themmrf.org**.

WHO GETS TREATED?

Generally, you won't receive treatment for myeloma until you develop symptoms.

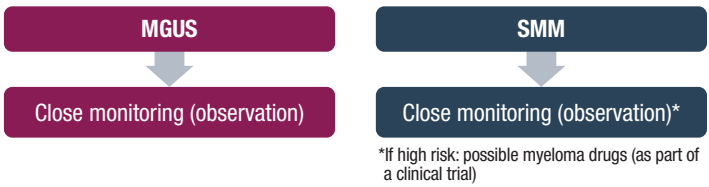
There are two **myeloma precursor conditions: monoclonal gammopathy of undetermined significance (MGUS)** and **smoldering multiple myeloma (SMM)**, in which **monoclonal (M) protein** is detectable in your blood and **clonal plasma cells** are present in your **bone marrow** but cause no symptoms or organ damage.

For more information on myeloma precursor conditions, refer to the companion booklet ***Multiple Myeloma Precursor Conditions*** and the MMRF website, **www.themmrf.org**.

If you have MGUS or SMM that does not progress, your doctor will monitor you as follows:

- MGUS: follow-up 6 to 12 months after diagnosis
- SMM: follow-up 2 to 3 months after diagnosis. If labs remain stable, this interval may be increased

Treatment approach to myeloma precursor conditions.



Patients with SMM or MGUS should be closely monitored through regular physical exams and blood and imaging tests. Patients should not receive treatment unless they develop **active multiple myeloma**.

Clinical trials are currently studying whether patients with high-risk SMM—that is, those who are at greater risk of rapidly progressing to **active multiple myeloma**—do better when they receive earlier treatment and also what type of treatment is best. Studies designed to identify MGUS or SMM earlier are also under way. Data collected from patients in these studies will help researchers identify clinical factors that may be associated with progression to active multiple myeloma.

WHAT FACTORS ARE CONSIDERED IN DEVELOPING A TREATMENT PLAN FOR ACTIVE MULTIPLE MYELOMA?

There is no one standard treatment for active myeloma. Each patient's treatment plan is based on a number of factors specific to him or her.

Your personal treatment plan: partnering with your health care team.

When you receive a diagnosis of active multiple myeloma, it's extremely important for you to commit to partnering with your doctor and health care team to review all of the factors of your disease and determine what treatment will work best. You should also share your treatment goals. Depending on the characteristics of the disease and your wishes, treatment plans may be designed to meet one or more goals.

In the MMRF, you have an advocate by your side—one who is an expert on all things myeloma, who is committed to helping you get the care and support you need, and who understands what you're going through. The Patient Navigation Center is available to answer your questions about disease management, treatments, clinical trials, and assistance with finding financial and other available resources.

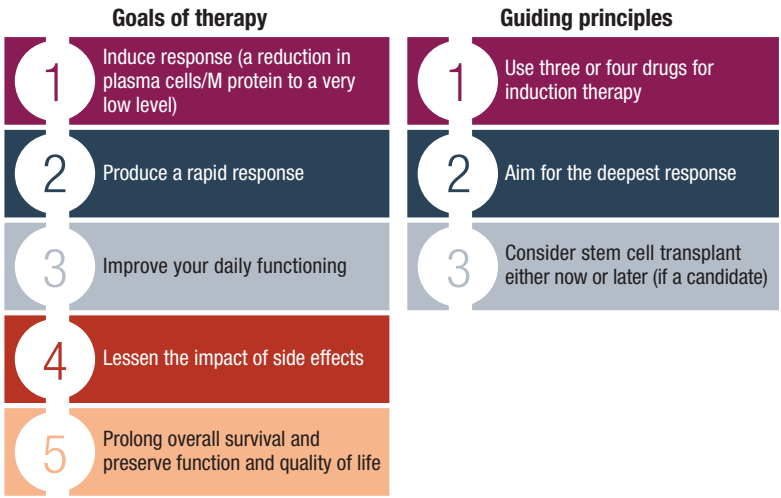
Telephone: **1-888-841-6673**
Monday–Friday, 9:00 AM to 7:00 PM ET
Email: **patientnavigator@themmrf.org**

GOALS OF MYELOMA THERAPY

The treatment of active multiple myeloma is usually aimed at reducing your symptoms and the number of myeloma cells in your bone marrow, which is determined by measuring the level of M protein in your blood. Achieving a response as quickly as possible while managing side effects is also a priority.

Most patients receive a four-drug (quadruplet) regimen as initial therapy (also called **induction therapy** or **frontline therapy**) following diagnosis. This treatment aims for as deep a response as possible (reducing plasma cells and M protein to a very low level). Some patients may be given three drugs (triplet) based on other health factors. This is typically followed by either high-dose chemotherapy and an **autologous stem cell transplant (ASCT)** or **maintenance therapy** using lower doses of some or all of the drugs used in the initial treatment. These principles are described later in this booklet.

Goals and guiding principles of myeloma therapy.



If one regimen stops working, another one can be used. There are many choices available today—and treatments continue to improve.

INDUCTION THERAPY OPTIONS

The choice of initial treatment depends on many factors, including the features of your myeloma, the anticipated risk of side effects, convenience for you, and the familiarity of the doctor with the given regimen. One of the first questions that must be answered, by both you and your doctor, is whether you are a candidate for ASCT.

If you're a candidate for ASCT, you may choose to have a transplant after three to six cycles of induction therapy. Or you may decide to complete induction therapy and consider transplant later, in which case your health care team would collect stem cells and store them for future use.

Clinical trials looking to determine the most appropriate duration of maintenance therapy are still ongoing. The specific characteristics of your myeloma, as well as your preferences and the doctor's perspective, are considerations that influence how long you receive a particular therapy.

Key questions to ask your health care team when preparing for induction therapy.

- What are my treatment choices?
- What are the risks and benefits of my treatment choices?
- What can I do to prepare for treatment?
- How will treatment affect my normal routine?
- What lab values and test results are important to track for a response or to monitor for side effects?
- Is there a clinical trial that might be better suited for my type of myeloma or prognosis?
- What resources are available for me and my family?
- What is the best way to get in touch with you for questions or emergencies?
- Should I ask for **genomic sequencing**?

QUADRUPLET/TRIPLET REGIMENS

Triplet regimens have been standard treatment for induction therapy for the last several years. However, in recent years, quadruplet regimens have emerged as a new treatment standard because of their superior effectiveness. Quadruplet regimens typically add an anti-CD38 **monoclonal antibody** like Darzalex or Sarclisa onto a triplet regimen backbone. Although quadruplet regimens can produce deeper responses and higher rates of minimal residual disease (MRD) negativity, they may cause more side effects.

Your doctor will consider several factors when deciding between triplet or quadruplet therapy. Doublets (two-drug combinations) may be considered when side effects are of particular concern.

Effective induction regimens for multiple myeloma.

Regimen	Abbreviation
Quadruplets	
Darzalex, Velcade, Revlimid, dexamethasone	D-VRd
Darzalex, Kyprolis, Revlimid, dexamethasone	D-KRd
Sarclisa, Velcade, Revlimid, dexamethasone	Isa-VRd
Sarclisa, Kyprolis, Revlimid, dexamethasone	Isa-KRd
Triplets	
Velcade, Revlimid, dexamethasone*	VRd
Kyprolis, Revlimid, dexamethasone	KRd
Darzalex, Revlimid, dexamethasone	DRd

*For patients with poor kidney function, cyclophosphamide is sometimes used in place of Revlimid (CyBorD).

REVLIMID

Revlimid (lenalidomide) is an **immunomodulatory drug** approved by the FDA for patients with active multiple myeloma, whether their disease is newly diagnosed, **relapsed**, or **refractory** (that is, it has recurred after initially responding to therapy or has progressed during therapy). Revlimid is also approved for use as maintenance therapy following ASCT. It's given orally and usually taken once a day. Cycles are typically 4 weeks, with treatment on days 1–21 and no treatment for days 22–28, though some clinical trials use continuous therapy (that is, treatment all 4 weeks) at lower doses. Doses can range from 2.5 mg to 25 mg, with patients typically starting at a higher dose and moving to a lower dose if they have a sustained response to treatment.

Revlimid (lenalidomide).

Current use(s)*	How is Revlimid administered?	What are the possible side effects?‡
• Newly diagnosed myeloma • Relapsed/refractory myeloma • Maintenance therapy following ASCT	• Oral capsule • For relapsed/refractory or newly diagnosed myeloma: 25 mg† once daily for 21 days out of a 28-day cycle (3 weeks on, 1 week off) • For myeloma maintenance therapy: 10 mg† once daily continuously for 28 days of repeated 28-day cycles	• Potential for blood clots • Reduced blood counts • Rash • Fatigue • Muscle pain or muscle cramping • Diarrhea • Small chance of second new cancers when given with melphalan

*Generic version available.
†Dose may be adjusted as needed.
‡Black box warnings:
- Embryo-fetal toxicity; Revlimid is available only through a restricted distribution program
- Hematologic toxicity
- Venous and arterial thromboembolism

Fatigue is a common side effect of Revlimid that can sometimes be managed by adjusting the dose or changing the time of day that the pill is taken.

Revlimid can also decrease blood counts. When this occurs, medications like **growth factors** are sometimes given to bring your blood counts up. You may develop a rash when taking Revlimid, sometimes (though not frequently) to an extent where it's necessary to stop taking the drug.

Also, Revlimid can increase the risk of blood clots. Because of this, your treatment will also include, at the very least, taking a baby aspirin every day to prevent blood clots. If you have other blood clotting risk factors (for example, if you previously developed a blood clot or are sedentary), you might need to take something stronger than aspirin, such as an injectable blood thinner like Lovenox or an oral blood thinner like Eliquis or Xarelto.

VELCADE

Velcade (bortezomib) was the first **proteasome inhibitor** to be approved by the FDA for patients with newly diagnosed, relapsed, or refractory active myeloma.

Velcade (bortezomib).

Current use(s)*	How is Velcade administered?	What are the possible side effects?
• Newly diagnosed myeloma • Relapsed/refractory myeloma	• 1.3, 1.0, or 0.7 mg/m^2 once or twice a week: – Injection under the skin (subcutaneous) – Intravenous (less common)	• Peripheral neuropathy • Low platelets: blood clotting problems • Gastrointestinal problems: nausea, diarrhea, vomiting, loss of appetite • Fatigue • Rash

*Generic version available.

Velcade is typically given as an injection under the skin (**subcutaneously**); it may be given **intravenously** in some instances (for example, if you experience an injection site reaction from a subcutaneous injection). Its most common side effects are gastrointestinal symptoms (for example, nausea or diarrhea), but these are usually mild. Velcade can lower your **platelet** count, but the effect does not usually last long. You may develop a rash and become fatigued when taking Velcade.

Another common side effect of Velcade is peripheral **neuropathy**, which is damage to the nerves that can produce numbness, tingling, and in some cases pain, with symptoms typically starting in the toes or fingertips. If you experience these symptoms, it's important to notify your doctor, as adjusting the dose can prevent the neuropathy from getting worse. There are also other treatments that can help reduce the discomfort or pain associated with neuropathy.

KYPROLIS

Kyprolis is another proteasome inhibitor used for patients with newly diagnosed, relapsed, or refractory active myeloma.

Kyprolis (carfilzomib).

Current use(s)	How is Kyprolis administered?	What are the possible side effects?
• Newly diagnosed myeloma • Relapsed/refractory myeloma	• Intravenously • Once weekly or twice weekly as a 10- or 30-minute infusion	• Fatigue • Anemia • Nausea • Low platelet count • Shortness of breath • Diarrhea • Fever • Hypertension • Cardiac symptoms • Headache • Infection

Common side effects of Kyprolis include nausea, diarrhea, fever, headache, infections, shortness of breath, and reductions in some blood cell counts. Peripheral neuropathy is rare and tends to be mild when it occurs.

Although uncommon, there's a risk of cardiovascular side effects with Kyprolis, including congestive heart failure. If you have a heart condition, you'll be evaluated to determine whether Kyprolis is an appropriate treatment. If you have any heart problems, your doctor will monitor you closely while you take Kyprolis.

Studies are ongoing to evaluate Kyprolis in combination with other myeloma drugs and to assess its potential for use in additional types of patients.

DARZALEX

Darzalex (daratumumab) is an anti-CD38 monoclonal antibody used to treat patients with newly diagnosed myeloma and those with relapsed or refractory myeloma.

Darzalex (daratumumab).

Current use(s)	How is Darzalex administered?*	What are the possible side effects?
• Newly diagnosed myeloma • Relapsed/refractory myeloma	• Intravenous injection • Subcutaneous injection† • Once a week for the first 8 weeks then every 2 weeks for 4 months, then monthly – The first prescribed dose may be split over 2 consecutive days	• Infusion reactions • Fatigue • Nausea • Back pain • Fever • Cough • Upper respiratory tract infection

*Dose schedule varies slightly depending on combination and **formulation**.
†Subcutaneous formulation is named Darzalex-Faspro.

The most common side effects included fatigue, low **red blood cell** and platelet counts, and nausea. Some patients in clinical trials experienced **infusion reactions** (chills and low-grade fever) while receiving the drug. For this reason, you will receive medications before and after administration of Darzalex to reduce your risk of these reactions.

SARCLISA

Sarclisa (isatuximab) is an anti-CD38 antibody that is typically given in combination with Pomalyst or Kyprolis and dexamethasone to patients with relapsed or refractory myeloma. It can also be given in different combinations to patients with newly diagnosed myeloma.

Sarclisa (isatuximab).

Current use(s)	How is Sarclisa administered?	What are the possible side effects?
• Newly diagnosed myeloma • Relapsed/refractory myeloma	• Intravenously • Once a week for the first 4 weeks then every 2 weeks thereafter • Premedication for infusion reactions	• Low numbers of white blood cells known as neutrophils (neutropenia) • Infusion-related reactions • Pneumonia • Upper respiratory tract infection • Diarrhea • Anemia • Low numbers of white blood cells known as lymphocytes (lymphopenia) • Low platelet counts (thrombocytopenia)

The most common side effects include infusion-related reactions, pneumonia, diarrhea, and low blood counts.

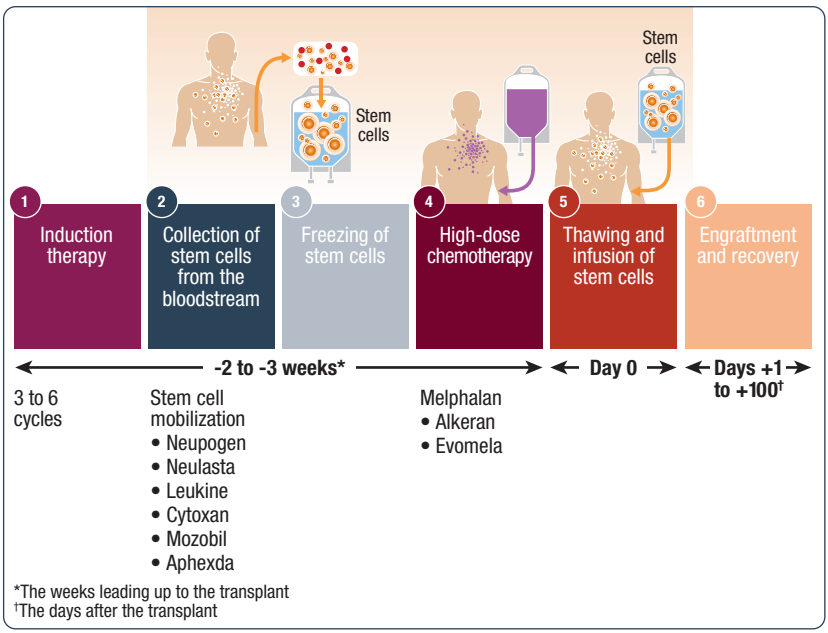
GENERICS

The FDA has approved generic versions of Revlimid (generic name: lenalidomide) and Velcade (generic name: bortezomib).

HIGH-DOSE CHEMOTHERAPY AND ASCT

High-dose chemotherapy (usually melphalan) with ASCT is a treatment that, for many patients with active myeloma, offers the best chance for long-lasting remission. High-dose chemotherapy, though effective in killing myeloma cells, also destroys normal blood-forming cells (called **hematopoietic stem cells**) in the bone marrow. Stem cell transplantation replaces these important cells. Results of this approach to myeloma therapy have improved with the release of several newer drugs.

Autologous stem cell transplant.



MAINTENANCE (OR CONTINUOUS) THERAPY

Myeloma is not yet curable, so it can recur even if you achieve a complete response. The goal of maintenance therapy is to maintain your initial response to treatment for as long as possible and hopefully improve survival.

Several **phase 3** trials have shown that Revlimid helps extend remission for patients following transplant. Revlimid is typically given until your myeloma progresses or you experience unacceptable side effects.

Low blood counts are commonly seen with Revlimid maintenance. If your blood counts get too low, it may be necessary for the doctor to reduce your dose. A small increase in second cancers (such as acute myeloid leukemia or various solid tumors), likely related to maintenance therapy and any doses of melphalan, was seen in all trials, but most researchers believe that the benefits likely outweigh the risks for most patients.

Several smaller (**phase 2**) trials have shown that maintenance therapy with Velcade can also improve outcomes. If you have high-risk myeloma or are unable to tolerate Revlimid, your doctor may recommend maintenance therapy with a different treatment (for example, Velcade).

Ninlaro, an oral drug in the same class as Velcade, was studied as maintenance therapy for patients following ASCT in a phase 3 trial. The results showed that some patients remained in remission longer on Ninlaro maintenance therapy (as compared to patients who received no maintenance therapy). Also, Ninlaro maintenance helped deepen the treatment response. If you are unable to tolerate Revlimid for an extended time, Ninlaro may be a suitable alternative.

The improvement seen in the length of time patients remain without relapse has prompted many doctors to discuss Revlimid maintenance therapy with their patients.

For high-risk patients, there's no standard treatment approach for maintenance, but treatment will usually be a combination of therapies (typically Revlimid plus at least one other agent, such as Velcade, Kyprolis, or Darzalex). Alternatively, high-risk patients are encouraged to enroll in a clinical trial.

As patients experience longer and deeper remissions with induction therapy followed by high-dose chemotherapy and transplant and/or maintenance therapy, it may be appropriate to lower doses of treatment or stop treatment altogether after several years of remission.

Maintenance therapy options.

Revlimid	• Reduction in myeloma progression • Improved survival (one of three studies, meta-analysis) • Increased risk of second cancers when used after melphalan • Approved for use as maintenance treatment after ASCT
Velcade-based treatment	• Supported by several smaller studies • Combined with Revlimid recommended for high-risk MM
Ninlaro	• Oral proteasome inhibitor • Reduction in myeloma progression (one large study)
Kyprolis/Revlimid	• Improved survival (one large study) compared with lenalidomide alone • Recommended for high-risk MM
Darzalex ± Revlimid	• Supported by two large studies • Two drug combination recommended for high-risk MM

Additional agents under investigation: Empliciti

HOW DO I KNOW IF A TREATMENT IS WORKING?

During and after treatment, doctors monitor your symptoms and may also perform some of the same tests that were done when you were initially diagnosed with active myeloma. The results of these tests show how well the treatment is working and may detect side effects. These tests also help determine if, after an initial response to treatment, your myeloma relapses.

The outcome of treatment in myeloma is defined using very specific standards or criteria. A stronger or deeper response is usually associated with better prognosis. However, you can do well even if you never achieve a complete response.

Response to treatment is defined using the following criteria:

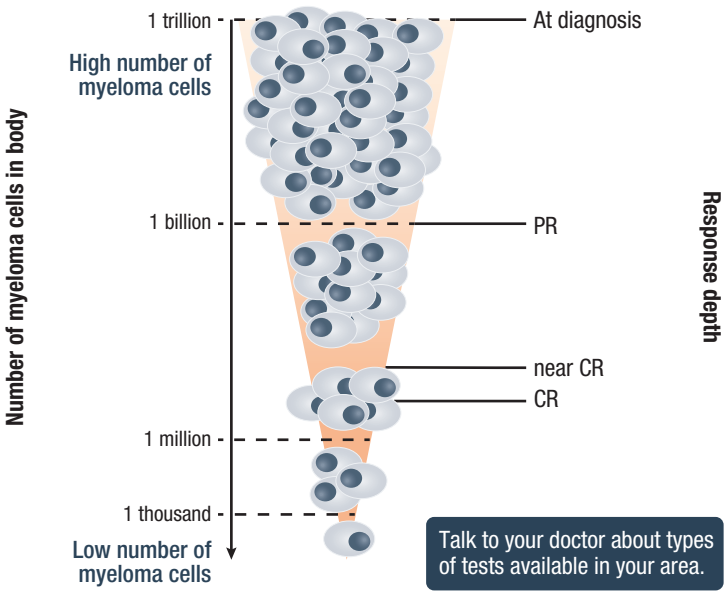
- Sustained MRD negativity
  - Multiple MRD negativity results as measured in the bone marrow; confirmed minimum of 1 year apart
- MRD negativity
  - Absence of myeloma cells in bone marrow samples
  - Typically evaluated by either **next-generation flow** or **next-generation sequencing**
- **Stringent complete response (sCR)**
  - A complete response plus normal **free light chain** ratio and absence of clonal cells in bone marrow as measured by immunohistochemistry (blood test)
- **Complete response (CR)**
  - Negative **immunofixation** in blood and urine samples
  - Disappearance of any soft **tissue plasmacytomas**
  - Less than 5% plasma cells in the bone marrow
- **Very good partial response (VGPR)**
  - M protein detectable in blood or urine by immunofixation (but not on **electrophoresis**), or
  - At least 90% reduction in M protein in blood plus M protein in urine to less than 100 mg per 24 h
- **Partial response (PR)**
  - At least 50% reduction in M protein in blood plus M protein in urine to less than 200 mg per 24 h (or reduction in 24-hour urinary M protein by at least 90%)
- **Minimal response (MR)**
  - At least 25% but no more than 49% reduction of M protein in blood and reduction in 24-hour M protein in urine by 50% to 89%
- **Stable disease (SD)**
  - Does not meet criteria for response or progressive disease
- **Progressive disease (PD)**
  - An increase of 25% in M protein
  - An increase of 10% in bone marrow plasma cells
  - An increase in M-protein in blood of at least 0.5 g/dL

For patients newly diagnosed with active myeloma, the goal of treatment is typically a VGPR or better. That is, you have no (or only a very small amount of) M protein detectable in your blood or urine. Luckily, with the treatments that are available today, more and more patients are achieving a CR.

WHAT IS MINIMAL RESIDUAL DISEASE (MRD)?

Treatment advances have increased the likelihood that you will achieve a CR. However, achieving a CR does not eliminate all myeloma in your body. Some myeloma cells can remain. This is called **minimal residual disease (MRD)** and is reported as MRD positive.

Minimal residual disease.



Conventional blood tests are not sensitive enough to detect these remaining cells, so MRD can only be measured from bone marrow. MRD measurement aims to detect any myeloma cells that remain in your body after a CR is achieved.

Studies using newer, more sensitive tests to detect MRD have shown that patients who achieve deeper responses with fewer remaining myeloma cells may have better outcomes. With today's therapies, more and more patients are achieving deep responses. Thus, interest in the assessment of MRD is growing.

An FDA-approved molecular test called the clonoSEQ assay is available to detect and monitor MRD in bone marrow samples from patients with active myeloma.

The extent of MRD positivity or negativity depends on the MRD test used and how sensitive it is in detecting myeloma cells in the sample (for example, one myeloma cell out of 100,000 normal cells or one myeloma cell out of 1,000,000 normal cells).

Currently, measurement of MRD depends on detecting myeloma cells in samples from your bone marrow and not other areas of your body. Therefore, imaging (for example, **positron emission tomography [PET]** or **computed tomography [CT]** scans) is also required to detect any myeloma cells that are present outside of your bone marrow. Also, it's premature to base treatment decisions on the results of MRD testing. For example, it's unclear whether patients who are MRD positive should get more treatment or if patients who are MRD negative no longer need treatment. Also, some patients never achieve MRD negativity and continue to live without major complications. MRD is an area of ongoing investigation in clinical trials.

WHAT ARE MY OPTIONS IF I RELAPSE OR IF I DON'T RESPOND TO THERAPY?

If you relapse or become refractory to therapy, you have the benefit of having many novel agents available as options for your treatment—including molecularly targeted and immunotherapeutic agents. In some cases, older treatments (such as older chemotherapies) may be appropriate, particularly if you don't respond to other agents.

FDA-approved myeloma drugs.

Immunomodulatory drugs	Revlimid (lenalidomide)	Pomalyst (pomalidomide)	
Proteasome inhibitors	Velcade (bortezomib)	Kyprolis (carfilzomib)	Ninlaro (ixazomib)
Chemotherapy alkylators	Cytoxan (cyclophosphamide)	Melphalan	
Steroids	Dexamethasone	Prednisone	
Monoclonal antibodies	Empliciti (elotuzumab)	Darzalex (daratumumab)	Sarclisa (isatuximab)
Bispecific antibodies	Elrexfio (elranatamab)	Talvey (talquetamab)	Tecvayli (teclistamab)
Other mechanism of action	XPOVIO (selinexor)		
CAR T-cell therapy	Abecma (idecabtagene vicleucel)	Carvykti (ciltacabtagene autoleucel)	

If your myeloma relapses or is refractory to treatment, several factors need to be taken into account to select a regimen that balances effectiveness and the risk of toxicity.

Factors to consider in choosing therapy for relapsed or refractory myeloma.

Many treatments are available for relapsed or refractory myeloma, and many potential new drugs are currently being studied. If your myeloma does not respond to induction therapy, or your disease progresses within 60 days after induction therapy is completed, your myeloma is considered to be refractory. However, if you are refractory to a particular drug, you may respond if the drug is used in combination with other myeloma medications.

Treatment options include:

- Any myeloma drug that has not been previously used
- A different combination of myeloma medications (which can include a previously used drug)
- High-dose chemotherapy and stem cell transplant (if appropriate)
- Participation in a clinical trial

To accelerate development of new therapies for myeloma, all eligible patients should consider participating in a clinical trial.

REVLIMID AND VELCADE REGIMENS

Treatment regimens in which Revlimid is combined with Velcade and dexamethasone (for example, D-VRd or VRd) may be options depending on whether you received them previously and how you responded.

Combining current and new drugs in development with treatment regimens based around Revlimid or Velcade continues to be evaluated in clinical trials.

PROTEASOME INHIBITORS

Ninlaro (ixazomib).

Current use(s)	How is Ninlaro administered?	What are the possible side effects?
• Relapsed/refractory myeloma	• Oral capsule	• Diarrhea • Constipation • Low platelet counts • Peripheral neuropathy • Nausea • Peripheral edema • Vomiting • Back pain

The most common side effects associated with Ninlaro include gastrointestinal effects (diarrhea, constipation, nausea, or vomiting), **thrombocytopenia**, and peripheral neuropathy. The most common serious side effects were thrombocytopenia and diarrhea.

Ninlaro is being evaluated in phase 3 trials in newly diagnosed myeloma in combination with Revlimid and dexamethasone and as maintenance therapy.

IMMUNOMODULATORY DRUGS

The drug listed below is in the same class as Revlimid.

Pomalyst (pomalidomide).

Current use(s)	How is Pomalyst administered?*	What are the possible side effects?†
• Relapsed/refractory myeloma	• Oral capsule	• Fatigue and weakness • Low white blood cell counts • Anemia • Gastrointestinal effects (constipation, nausea, or diarrhea) • Shortness of breath • Upper respiratory infection • Back pain • Fever • Blood clots

*Dosing can be adjusted if needed.
†Black box warnings:
- Embryo-fetal toxicity; Pomalyst is available only through a restricted distribution program
- Venous and arterial thromboembolism

Side effects vary by patient and are considered manageable. The most common include fatigue and loss of strength, low white cell blood counts, **anemia**, constipation, nausea, diarrhea, shortness of breath, upper respiratory tract infections, back pain, and fever. Similar to other immunomodulatory drugs, some patients who received Pomalyst in clinical trials developed blood clots. For this reason, aspirin or another blood thinner is given with Pomalyst.

Pomalyst is used in combination with dexamethasone and certain monoclonal antibodies as a treatment for some myeloma patients.

Numerous clinical trials are continuing to evaluate the use of Pomalyst in other types of patients and in combination with other myeloma drugs.

BISPECIFIC ANTIBODIES

Bispecific antibodies are another type of **antibody**-based **immunotherapy** and are made by fusing together fragments from two regular antibodies—like those normally produced by your **immune system**. One fragment attaches to proteins on the myeloma cells (making them easier for your immune system to find). The other fragment attaches to proteins found on your immune cells—specifically, **T cells**—and helps these T cells find and fight the tagged myeloma cells.

Tecvayli

Tecvayli (teclistamab) is a BCMA–directed bispecific antibody. Tecvayli targets both BCMA on the surface of multiple myeloma cells and CD3 receptors expressed on the surface of T cells.

The most common side effects included **cytokine release syndrome**, fever, low blood counts, and musculoskeletal pain.

Tecvayli (teclistamab).

Current use(s)	How is Tecvayli administered?	What are the possible side effects?†
• Relapsed/refractory myeloma	• Subcutaneous injection • Two **step-up* doses** on day 1 and day 4, followed by the first full treatment dose on day 7; Tecvayli is given once weekly thereafter until disease progression or unacceptable toxicity • Patients who have achieved and maintained a complete response or better for at least 6 months may have dosing frequency decreased to every two weeks until disease progression or unacceptable toxicity. • Hospitalization required for each step-up dose and first full treatment dose	• Cytokine release syndrome • Neurotoxicity • Infection • Low blood counts • Fever • Musculoskeletal pain • Injection site reaction • Fatigue

*Step-up doses are smaller initial doses that gradually increase to the full dose to minimize adverse effects.
†Black box warnings:
- Cytokine release syndrome
- Neurologic toxicities
- Tecvayli is available only through a restricted distribution program

Elrexfio

Elrexfio (elranatamab) is a BCMA-directed bispecific antibody that targets both BCMA on the surface of multiple myeloma cells and CD3 receptors on the surface of T cells.

The most common side effects associated with Elrexfio are cytokine release syndrome, low blood counts, fatigue, and infection.

Elrexfio (elranatamab).

Current use(s)	How is Elrexfio administered?	What are the possible side effects?*
• Relapsed/refractory myeloma	• Subcutaneous injection • Two step-up doses on days 1 and 4, followed by the first full treatment dose on day 8; Elrexfio is given once weekly thereafter • After 24 weeks, patients who are responding to therapy may have dosing frequency decreased to every 2 weeks • Hospitalization required for 48 hours after first step-up dose and 24 hours after second step-up dose	• Cytokine release syndrome • Neurotoxicity • Infection • Low blood counts • Musculoskeletal pain • Injection-site reaction • Fatigue

*Black box warnings:
- Cytokine release syndrome
- Neurologic toxicity
- Elrexfio is available through a restricted distribution program

Talvey

Talvey (talquetamab) is a GPRC5D–directed bispecific antibody that targets both GPRC5D on the surface of multiple myeloma cells and CD3 receptors on the surface of T cells.

The most common side effects associated with Talvey are cytokine release syndrome, altered sense of taste (**dysgeusia**), nail disorders, and fever.

Talvey (talquetamab).

Current use(s)	How is Talvey administered?	What are the possible side effects?*
• Relapsed/refractory myeloma	• Subcutaneous injection • Two step-up doses on days 1 and 4, followed by the first full treatment dose on day 7; Talvey is given once weekly thereafter • Alternatively, three step-up doses on days 1, 4, and 7 followed by the first full treatment dose on day 10; Talvey is given every 2 weeks thereafter • Hospitalization required for 48 hours after all doses within the step-up dosing schedule	• Cytokine release syndrome • Neurotoxicity • Infection • Altered sense of taste • Low blood counts • Musculoskeletal pain • Injection site reaction • Fatigue • Nail changes • Fever

*Black box warnings:
- Cytokine release syndrome
- Neurologic toxicity
- Talvey is available through a restricted distribution program

CAR T-CELL THERAPY

Immune cell therapy is the process of extracting your own immune cells, engineering them in a laboratory to be better able to identify and attack myeloma cells, and then returning them to you.

Abecma

Abecma (idecabtagene vicleucel) is a first-in-class BCMA-directed personalized immunotherapy called **chimeric antigen receptor (CAR) T-cell therapy**. Abecma is manufactured using T cells that have been collected from your blood. The T cells are modified in a laboratory to recognize BCMA, a protein expressed on multiple myeloma cells. CAR T cells are then infused back into you—now with an enhanced ability to find and kill myeloma cells.

Abecma (idecabtagene vicleucel).

Current use(s)	How is Abecma administered?	What are the possible side effects?*
• Relapsed/refractory myeloma	• Intravenously • One-time infusion 2 days after completing lympho-depleting chemotherapy (cyclophosphamide IV and fludarabine) • Hospitalization required for treatment infusion	• Low blood counts • Cytokine release syndrome • Neurotoxicity • Infection • Fatigue • Musculoskeletal pain • Hypogammaglobulinemia • Diarrhea

*Black box warnings:
- Cytokine release syndrome
- Neurologic toxicities
- Hemophagocytic lymphohistiocytosis/ macrophage activation syndrome
- Prolonged cytopenia
- T-cell malignancies
- Abecma is available only through a restricted distribution program

The efficacy of Abecma was studied in a clinical trial of 100 patients with relapsed or refractory myeloma who received CAR-positive T cells. Patients in this study had received three to sixteen previous therapies, with most patients receiving six. Nearly all had received a previous ASCT. Treatment response lasted around 20 months.

The most common side effects included low blood counts, cytokine release syndrome, **neurotoxicity**, infection, and fatigue.

Carvykti

Carvykti (ciltacabtagene autoleucel) is another CAR T-cell therapy that uses T cells that have been modified to recognize BCMA.

The efficacy of Carvykti was studied in a clinical trial of 97 patients with relapsed or refractory myeloma who received CAR T cells. Patients in this study had received four to eight previous therapies, with most patients receiving six. Treatment response lasted greater than 2.5 years.

The most common side effects include low blood counts, cytokine release syndrome, fever, low blood pressure, and **hypogammaglobulinemia**.

Carvykti (ciltacabtagene autoleucel).

Current use(s)	How is Carvykti administered?	What are the possible side effects?*
• Relapsed/refractory myeloma	• Intravenously • One-time infusion after completing lymphodepleting chemotherapy (cyclophosphamide IV and fludarabine IV daily for 3 days) • Hospitalization required after treatment infusion	• Low blood counts • Cytokine release syndrome • Neurotoxicity • Infection • Fever • Musculoskeletal pain • Fatigue • Hypogammaglobulinemia • Hypotension

*Black box warnings:
- Cytokine release syndrome
- **Immune effector cell-associated neurotoxicity syndrome (ICANS)**
- Parkinsonism and Guillain-Barré syndrome and associated complications
- Hemophagocytic lymphohistiocytosis/ macrophage activation syndrome
- Prolonged and/or recurrent cytopenias
- Secondary hematologic malignancies, including myelodysplastic syndrome and acute myeloid leukemia
- Carvykti is available only through a restricted distribution program

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For more information about the different types of immunotherapy, refer to the companion booklet ***Multiple Myeloma Immunotherapy*** and the MMRF website, **themmrf.org**.

MONOCLONAL ANTIBODIES

Empliciti (elotuzumab).

Current use(s)	How is Empliciti administered?	What are the possible side effects?
• Relapsed/refractory myeloma	• Intravenous injection • Once a week for the first 8 weeks then every 2 weeks	• Fatigue • Diarrhea • Fever • Constipation • Cough • Peripheral neuropathy • Infusion reactions • Nasopharyngitis • Upper respiratory tract infection • Decreased appetite • Pneumonia • Small chance of second new cancer

The most common side effects included fatigue, diarrhea, fever, constipation, cough, infection of the nose and throat (nasopharyngitis), upper respiratory tract infection, pneumonia, peripheral neuropathy, and decreased appetite.

OTHER MECHANISMS OF ACTION

Drugs with other **mechanisms of action** work in different ways than drugs in the other classes. Myeloma drugs with novel mechanisms of action target proteins involved in cell growth and division. These drugs may target proteins that are specific to myeloma cells or common to all cells.

Xpovio

Xpovio (selinexor) is the first in a drug class called nuclear export inhibitors. Xpovio targets—and disrupts the function of—a protein called XPO1, which ultimately leads to myeloma cell death.

Xpovio (selinexor).

Current use(s)	How is Xpovio administered?	What are the possible side effects?
• Relapsed/refractory myeloma	• Oral tablet • Taken once or twice a week	• Low platelet count • Low white blood cell counts • Fatigue • Anemia • Decreased appetite • Decreased weight • Diarrhea • Vomiting • Low sodium levels • Constipation • Shortness of breath • Upper respiratory infection

The most common side effects associated with Xpovio include diarrhea, nausea and vomiting, fatigue, and reductions in platelets, **white blood cells**, and red blood cells.

CHEMOTHERAPY

Chemotherapeutic agents like cyclophosphamide and bendamustine are still commonly used in relapsed or refractory myeloma. Chemotherapy-based regimens that are used include the following:

Chemotherapy regimens for multiple myeloma.

Regimen	Abbreviation
Velcade, cyclophosphamide, dexamethasone	CyBorD
Kyprolis, cyclophosphamide, dexamethasone	CCyd
Darzalex, cyclophosphamide, bortezomib, dexamethasone	D-VCd
Ninlaro, cyclophosphamide, dexamethasone	ICd
Revlimid, cyclophosphamide, dexamethasone	CRd
Kyprolis, cyclophosphamide, thalidomide, dexamethasone	CYKLONE
Dexamethasone, cyclophosphamide, etoposide, cisplatin	DCEP
Dexamethasone, thalidomide, cisplatin, doxorubicin, cyclophosphamide, etoposide with or without bortezomib	VTD-PACE or DT-PACE, respectively
Bendamustine	

BEYOND TREATMENT: MAINTAINING A HEALTHY LIFESTYLE

In addition to following the treatments you and your health care team choose, there are things you can do to maintain your overall health and maintain (or improve) your quality of life as you navigate living with myeloma.

NUTRITION

Maintaining good nutrition can have important benefits for myeloma patients. Eating a healthy diet can help keep your immune system in peak shape, which helps you avoid infections (or at least recover from them faster).

Some myeloma treatments, like high-dose chemotherapy, can reduce your appetite, so it's important that you make the most of what you eat. Eating small meals every few hours and keeping energy-dense high-protein snacks like nuts, eggs, and cheese handy are two strategies that can help you maintain a healthy diet.

Your nutritional needs may have changed since your myeloma diagnosis, and they may continue to change throughout your treatment. Your care team may recommend that you meet with a nutritionist to review your diet plan and recommend supplements if needed.

EXERCISE

Getting regular exercise can improve your physical and mental health while you receive myeloma treatment, providing such benefits as maintaining fitness, strengthening bones, boosting your immune system, and reducing fatigue.

How much exercise is "enough" will depend on your fitness level and where you are in your myeloma journey. For example, weight-bearing exercise may not be an option if you are at a higher risk for bone fractures.

Before starting any kind of exercise, it's important to talk with your care team.

MENTAL HEALTH AND EMOTIONAL SUPPORT

Multiple myeloma often causes fear, anxiety, and distress. Many sources of support are available, including caregivers, mental health professionals, and myeloma support groups.

Stress-reducing activities can also help reduce anxiety and improve your mental well-being; some options include acupuncture, deep-breathing exercises, massage, meditation, mindfulness, and yoga.

As with physical exercise, it's important to keep a dialogue going with your care team about what strategies you use to manage the mental and emotional challenges of multiple myeloma.

The MMRF's Patient Navigation Center is a resource where patients and caregivers can connect (by phone, email, or online) with patient navigators who can offer support. In addition, it may help to speak to another patient or caregiver who has been through the same experience. Myeloma Mentors allows patients and caregivers the opportunity to connect with a trained patient and/or caregiver mentor to share his or her journey and experience.

Contact the Patient Navigation Center at **888-841-6673** to be connected to a patient navigator or a Myeloma Mentor, or visit **themmrf.org** to learn more.

SLEEP AND FATIGUE

For many myeloma patients, sleep disturbances are common and can result from a number of causes, including pain, medication, depression, and anxiety. Many myeloma drugs can cause insomnia, daytime sleepiness, fatigue, and/or side effects that are worse at night, which can make getting restful sleep more challenging.

Determining the cause of any sleep disturbances you experience can help your care team devise strategies for reducing them.

Fatigue—a feeling of persistent tiredness, weakness, and lack of energy—is another challenge that you may encounter, as it's a common effect of both multiple myeloma itself and many of the treatments used to treat it. If you're having difficulty sleeping, it's more likely that you will feel fatigued.

IS A CLINICAL TRIAL RIGHT FOR ME?

Clinical trials are essential to the development of new myeloma treatments, providing new therapeutic options for myeloma patients at all stages of the disease. The greater the number of people there are enrolling in clinical trials, the faster new treatments can be made available to patients. It's only through patient participation in clinical trials that we have achieved the high number and various types of myeloma treatments available today.

Clinical trials compare new treatments or combinations with current standards of care. If you enroll in a clinical trial, you have the opportunity to be among the first to receive the newest drugs and therapies in development—before they're available commercially.

However, it's important to understand that new treatments may be equivalent to, more effective than, or not as effective as standard treatment options. They may also have unexpected side effects.

Before any drug is considered for testing in people, evidence of activity against the disease must have been demonstrated in laboratory and animal studies—these are called **preclinical studies**.

In all myeloma clinical trials, participants receive either the experimental therapy being tested or the best available standard treatment. In other words, you will never receive no treatment in a myeloma clinical trial.

Clinical trials take place in different stages, with each phase serving a distinct purpose.

Clinical trial stages.

	Phase 1	Phase 2*	Phase 3†
Objectives	Optimal dose Side effects Metabolism	Preliminary effectiveness Additional safety	Definitive effectiveness and safety
Treatment	Single arm (all patients receive experimental therapy)	Single arm Two arms of different treatments or doses: patients randomly assigned to an arm	Two arms: patients randomly assigned to receive experimental therapy or standard therapy
Typical study size	Small (<50)	Varies	>200

*When no standard treatment is available, FDA may approve drugs based on trial results.
†Conducted to receive FDA approval of new drugs, in most cases.

Based on the results of clinical trials, the FDA approves treatments that are safe, effective, and shown to be better than the standard treatments available.

Clinical trials take place at cancer centers, hospitals, clinics, or doctors' offices. Before you enroll in a clinical trial, all details of the treatment are explained, and you must consent to participate. If you agree to participate in a clinical trial, you're free to withdraw at any time.

FINDING A CLINICAL TRIAL

The MMRF Patient Navigation Center is designed to match patients with appropriate clinical trials. To take advantage of this program, you (or your caregiver or family member) can complete a simple questionnaire online at **themmrf.org/resources/clinical-trial-finder**. Or you can call 888-841-6673 to speak with an MMRF patient navigator, who will ask you questions and talk to you about clinical trials in your area or ones that may be appropriate for you.

How do I find a clinical trial?

1. Ask your treating hematologist or oncologist about any available trials
2. Check with any academic medical centers close to your home
3. Search for a clinical trial in your area or let an MMRF patient navigator help guide you through the process at **themmrf.org/resources/clinical-trial-finder**

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, Michael Riotta of Jamison, 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 todayy.

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

REIMBURSEMENT-ASSISTANCE PROGRAMS

Patient Access Network
Website: www.panfoundation.org
Services: Help and hope to people with chronic or life-threatening illnesses for whom cost limits access to critical medical treatments
Phone: 1-866-316-PANF (1-866-316-7263)
Contact: www.panfoundation.org/contact

Amgen Inc
Products: Neupogen/Neulasta/Kyprolis/Xgeva
Website: www.amgensupportplus.com/patient
Phone: 1-866-264-2778

Bristol-Myers Squibb
Products: Empliciti/Pomalyst/Revlimid/Thalomid/Abecma
Website: www.bmsaccesssupport.com/patient
Phone: 1-800-861-0048

Janssen
Product: Darzalex
Website: www.janssencarepath.com/patient/darzalex/cost-support
Phone: 1-877-227-3728

Product: Carvykti
Website: www.carvykti.com/resources-and-support
Phone: 1-800-559-7875

Product: Talvey
Website: www.talvey.com/support-and-resources/#janssen-compass
Phone: 1-844-628-1234

Product: Tecvayli
Website: www.janssencarepath.com/patient/tecvayli/patient-support
Phone: 1-877-227-3728

Karyopharm
Product: XPOVIO
Website: www.karyforward.com
Phone: 1-877-KARY4WD (1-877-527-9493)

Pfizer
Product: Elrexfio
Website: https://www.elrexfio.com/navigating-support-financial-resources
Phone: 1-877-744-5675

Sanofi
Product: Sarclisa
Website: www.sanoficareassist.com
Phone: 1-833-WE+CARE (1-833-930-2273)

Takeda Oncology Company
Product: Velcade/Ninlaro
Website: www.here2assist.com/patient/home
Phone: 1-844-817-6486, Option 2

GLOSSARY

active multiple myeloma Multiple myeloma in which the percentage of plasma cells in the bone marrow is greater than 10% and in which the patient shows one or more CRAB symptoms (see *CRAB*)

adaptive immunity Part of the immune system that is composed of highly specialized cells designed to recognize foreign invaders and attack them any time they enter the body

anemia Decrease in the number of red blood cells in the blood

antibody Protein produced by plasma cells that helps protect the body from infection and disease (also called *immunoglobulin*; see also *monoclonal antibody*)

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

bispecific antibody Monoclonal antibody that can bind to two different cell surface proteins at the same time

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

chimeric antigen receptor T (CAR-T) cell therapy A form of immunotherapy in which a patient's immune cells (mostly T cells) are collected, engineered in a lab to be better able to identify and attack myeloma cells, and then returned to the patient; examples are Abecma and Carvykti

clinical trial Study of the safety and effectiveness of a therapeutic agent using consenting human participants

clonal Derived from a single mutated cell

complete response (CR) Treatment outcome in which the level of plasma cells in the bone marrow is no more than 5%, there is no evidence of myeloma proteins in the serum or urine as measured by standard laboratory techniques, and all signs and symptoms of cancer have disappeared (though cancer still may be in the body)

computed tomography (CT) Imaging technique that uses a computer to generate three-dimensional x-ray pictures

CRAB Acronym for the following group of clinical indicators of organ damage: increased calcium level, renal (kidney) failure, anemia, bone lesions; the presence of one or more of these indicators can help establish a diagnosis of multiple myeloma

cytokine release syndrome Common, flu-like side effect following infusion of CAR T cells in which a patient experiences fevers, chills, and low blood pressure

dysgeusia Condition that affects the sense of taste, causing foods to taste metallic, foul, rancid, or salty

electrophoresis Laboratory test used to measure the levels of proteins in the blood or urine; uses an electrical current to sort proteins by their charge

formulation The preparation of a drug

free light chain Short protein (immunoglobulin light chain) produced by myeloma cells and found in the blood

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

genomic sequencing Study of DNA sequencing of myeloma cells to detect mutations and to see how that DNA changes over time

growth factor Substance that stimulates cells to multiply

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

hypogammaglobulinemia Condition in which the levels of serum immunoglobin or antibodies in the body are reduced

immune effector cell–associated neurotoxicity syndrome (ICANS) Side effect of the nervous system that is commonly seen with certain immunotherapies (such as CAR T-cell therapy and bispecific antibody therapy) and can include confusion or delirium, speech problems (expressive aphasia), motor weakness, tremor, headache, seizures, and reduced level of consciousness

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

immunofixation Test to measure *immunoglobulins* in blood

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

immunomodulatory drugs Drugs that fight cancer by altering the function of the immune system; examples include Thalomid, Revlimid, and Pomalyst

immunotherapy Prevention or treatment of disease with drugs that stimulate the immune system

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*)

infusion reaction Symptoms that sometimes develop after a patient receives intravenous drugs; commonly include chills, fever, nausea, weakness, headache, skin rash, and/or itching; although rare, severe reactions such as difficulty breathing or low blood pressure can occur

intravenous Administration of a drug directly into a vein

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

mechanism of action Biochemical process through which a drug produces an effect on the body

minimal 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

minimal response (MR) Treatment outcome where there is less than a 50% decrease in M protein

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

monoclonal gammopathy of undetermined significance (MGUS) Condition that can occur before a patient develops or shows any symptoms of cancer; indicated by the presence of M protein in the serum or urine, MGUS may eventually progress to myeloma

monoclonal (M) protein Abnormal antibody found in large quantities in the blood and urine of individuals with myeloma

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

myeloma precursor condition A preceding phase of active multiple myeloma characterized by changes in the cells of the bone marrow but no symptoms or organ damage; see also *monoclonal gammopathy of undetermined significance (MGUS)*, *smoldering multiple myeloma (SMM)*

neuropathy Disorder of the nerves that can disrupt sensation or cause burning/tingling; when the hands and feet are affected, it is referred to as *peripheral neuropathy*

neurotoxicity Damage to nervous system including brain and/or nerves

next-generation flow Highly sensitive test that uses bone marrow samples to detect minimal residual disease

next-generation sequencing Highly sensitive test that uses genomic assessment of bone marrow samples to detect minimal residual disease

partial response (PR) Treatment outcome where there is a greater than 50% decrease in M protein and disappearance of some (but not all) signs and symptoms of cancer

phase 1 The first round of a clinical trial, conducted with a small number of participants to assess a drug's safety and non-toxic dosage levels

phase 2 The second stage of a clinical trial, conducted with a larger number of participants to assess a drug's effectiveness and further evaluate its safety

phase 3 The most advanced stage of drug development, conducted with a large number of participants to confirm a drug's effectiveness, identify and monitor its side effects, compare it to commonly used treatments, and collect information that will allow the drug to be used safely; usually required for FDA approval of drugs

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

plasmacytoma Tumor made up of cancerous plasma cells that occurs in bone or soft tissue; patients with a plasmacytoma may develop multiple myeloma

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

positron emission tomography (PET) Imaging technique in which radioactive glucose (sugar) is used to highlight cancer cells

preclinical studies Experiments conducted in the laboratory and in animals to identify a target for therapy and to confirm its anti-cancer activity

progressive disease (PD) Active myeloma that is worsening; in most cases, relapsed and/or refractory disease can be considered to be progressive disease

prognosis Prediction of the course and outcome of a disease

proteasome inhibitors Drugs that slow myeloma cell growth and kill myeloma cells by interfering with processes that play a role in cell function; examples include Velcade, Ninlaro, and Kyprolis

red blood cell Blood cell that carries oxygen

refractory Disease that progresses during therapy

relapsed Disease that progresses after initially responding to therapy

smoldering multiple myeloma (SMM) Condition characterized by increased M protein and slightly increased numbers of plasma cells in the bone marrow and an absence of symptoms; patients with SMM are monitored and only treated if their disease progresses; about 5% of myeloma patients have SMM

stable disease (SD) Treatment outcome in which the disease has not responded to therapy but has not progressed; also refers to disease that initially responded to therapy and remains stable after treatment is stopped

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

step-up dosing Method of giving a drug treatment in doses that start small and gradually increase to a full dose to minimize side effects

stringent complete response (sCR) Treatment outcome in which there are no detectable abnormal plasma cells in the bone marrow or M protein in the serum or urine and in which free light chain ratio test is normal

subcutaneous Administration of a drug under the skin

T cell (or T lymphocyte) Type of white blood cell that can be subdivided into two main groups called helper and cytotoxic T cells; helper T cells are responsible for *adaptive immunity*; cytotoxic T cells are killers of cells that have been targeted for death

thrombocytopenia Decrease in the number of platelets (small cell fragments in the blood that help it to clot)

tissue A group of structurally and functionally similar cells

very good partial response (VGPR) Treatment outcome in which there is a greater than 90% decrease in M protein

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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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

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Email: **patientnavigator@themmrf.org**

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