

# FACTSHEET

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MYELOMA PATIENTS EUROPE

CILTACABTAGENE AUTOLEUCEL  
OR CILTA-CEL (CARVYKTI®)



**MPE**  
Myeloma  
Patients  
Europe

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# FACTSHEET

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## MYELOMA PATIENTS EUROPE

Myeloma Patients Europe (MPE) has developed a series of factsheets for patients and patient advocates, providing an overview of available treatment options for myeloma and covering some topics related to the disease.

The factsheets cover important issues around the treatment, so that patients can feel safe and ask their doctor specific questions.

For each of the available therapies, the following topics will be addressed:

- What is myeloma?
- What is the particular treatment?
- How does the treatment work?
- What are the benefits?
- What are the side-effects?
- How and when is the treatment given?

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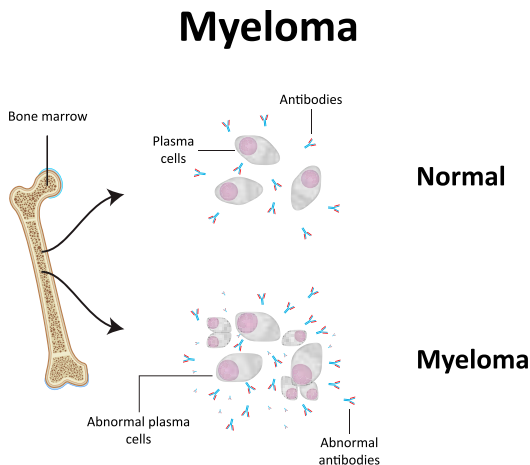
Myeloma treatment is constantly evolving and the factsheets will be updated regularly to reflect the latest developments.

# What is myeloma?

Myeloma is a rare cancer of the bone marrow that causes the formation of abnormal plasma cells, known as cancerous myeloma cells, which divide uncontrollably. Usually, plasma cells help the body fight infections by making antibodies that recognise and attack viruses, bacteria, and other cancer cells. Myeloma affects multiple places in the body (this is why it is sometimes referred to as 'multiple myeloma') where bone marrow is normally active, such as the bones of the spine, pelvis, rib cage, and the areas around the shoulders and hips.

Myeloma causes pain, anaemia (low red blood cells), fatigue, fractures, recurring infections, bruising, and high blood calcium (hypercalcaemia). These symptoms usually require treatment. After treatment, most myeloma symptoms may subside and not require treatment (also known as remission). Many patients may also be on treatment during remission to ensure their myeloma is kept at bay (known as maintenance treatment). After some time, the patient's symptoms may reappear, requiring treatment (also known as relapse). Myeloma usually occurs with periods of remission and relapse, and after some time, the patient's disease no longer responds to treatment (known as refractory myeloma).

Treatment may involve a combination, often three or four drugs, which is considered more effective than a single drug. Myeloma generally cannot be cured, but survival rates are increasing due to the availability of new treatments, and many patients can enjoy a good quality of life. Several new therapies have recently been approved or are under consideration for use following relapse or refractory myeloma.



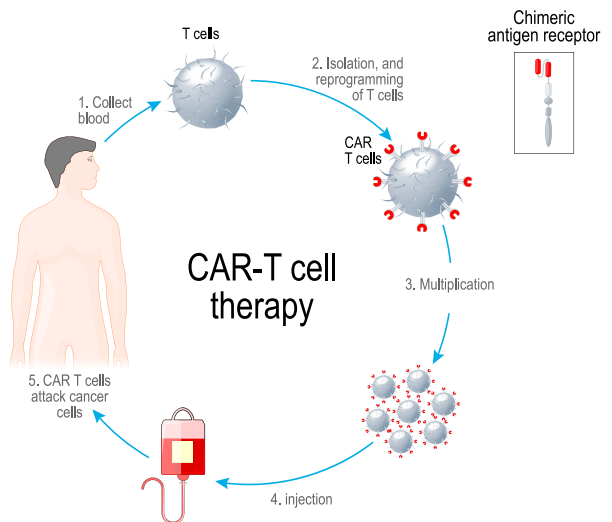
# What is ciltacabtagene autoleucl, cilta-cel (Carvykti®)?

Ciltacabtagene autoleucl (also known as cilta-cel or Carvykti®) is a chimeric antigen receptor T-cell therapy (CAR T-cell therapy). Cilta-cel was granted “orphan drug” designation by the European Commission in 2020. An orphan drug designation is a status assigned to medicines developed for rare disease conditions that affect fewer than five patients per 10,000 inhabitants in the EU.

The EMA authorised cilta-cel in May 2022 for treating adults with multiple myeloma whose cancer has returned (relapsed) or has not responded to treatment (refractory). Cilta-cel is used when patients' disease worsens despite receiving at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody<sup>1</sup>.

## How does ciltacabtagene autoleucl work?

Cilta-cel is a type of immunotherapy that uses a patient's white blood cells, specifically T cells, extracted through a blood draw. The T cells are then sent to a laboratory for manufacturing and genetic modification to include a protein molecule called a “chimeric antigen receptor” (CAR). The CAR protein sits on the surface of T cells to help find myeloma cells and kill them. Once the manufactured T cells are multiplied and reinfused into the patient's body, they find and bind to cancerous myeloma cells via the B cell maturation antigen (BCMA) protein often found on the surface of the myeloma cell. After binding, the T cells release cytokines to kill the myeloma cell and recruit the immune system.



## What are the benefits of ciltacabtagene autoleucl?

The safety and efficacy of cilta-cel were evaluated in the CARTITUDE-1 phase 1b/2 study in 113 patients with relapsed/refractory multiple myeloma, 97 of whom received cilta-cel. This study found that 98% of patients responded to treatment with cilta-cel, with a response lasting around 21.8 months<sup>2,3</sup>. A total of 61 patients who participated in the trial were measured for minimal disease negativity (also known as MRD; a test result that means no myeloma cells are detected in 100 thousand or even one million cells). In the CARTITUDE-1 trial, 91.8% of patients were MRD negative after treatment.<sup>2</sup>

Other clinical studies are ongoing to assess efficacy and safety of cilta-cel in broader populations of myeloma patients. For example, the CARTITUDE-2 study evaluated the efficacy and safety of cilta-cel in patients with multiple myeloma and early relapse after initial treatment. Of the 18 patients who received cilta-cel, the overall response rate was 88.9%<sup>4</sup>. Of the nine evaluated patients, 100% were MRD-negative after treatment.

## How is ciltacabtagene autoleucl given to patients?

First, you will undergo leukapheresis or white blood cell (T cell) extraction through a blood draw. These cells will be used to prepare the CAR T-cells. The manufacturing time for cilta-cel may take 4-6 weeks, during which time you might receive a bridging therapy (a combination treatment of standard myeloma drugs given to prevent disease progression before CAR T-cell infusion).

Before receiving cilta-cel, you will receive conditioning chemotherapy (also known as lymphodepletion therapy) to reduce your level of white blood cells and help your body accept the CAR T-cell therapy. When the CAR T-cells are ready, they are given through an infusion into a vein. Because of the potential side effects, infusions are administered in a certified healthcare facility where you can be monitored closely for at least seven days. After this, you will be instructed to stay within close proximity of a healthcare facility, or hospital, for four weeks for any potential side effects to be promptly managed. Conditioning treatment and CAR T-cell therapy may increase your risk of infection, therefore you may be advised to take precautions to prevent infection such as handwashing, wearing a mask and avoiding large crowds. Your provider may also recommend that you be up to date on vaccinations before treatment.

## What are the side effects of ciltacabtagene autoleucl?

The safety of cilta-cel was evaluated in the CARTITUDE-1 study of 97 patients. The most common other side effects of cilta-cel (occurring in more than 20% of patients, listed from most common to least common) included<sup>1,5</sup>:

- Fever (pyrexia)
- Cytokine release syndrome (CRS; a potentially life-threatening systemic inflammatory condition)
- Hypotension (low blood pressure)
- Pain in the muscles and bones
- Fatigue
- Infections
- Cough
- Chills
- Gastrointestinal symptoms (e.g., diarrhoea, nausea, decreased appetite)
- Encephalopathy (confusion)
- Upper respiratory tract infections
- Headache
- Tachycardia (a faster than normal heartrate)
- Dizziness
- Shortness of breath (dyspnoea)
- Fluid retention (oedema)
- Viral infections

Changes in blood electrolyte levels such as calcium, potassium and phosphate can also occur, although these are less common.

In addition to the above side effects, changes in blood levels were among the most common, including the following, which occurred in greater than 10% of patients:

- Lymphopenia and leukopenia (low levels of lymphocytes and other white blood cells, which increases the risk of infection)
- Neutropenia (low levels of a type of white blood cell called a neutrophil, which increases the risk of infection)
- Anaemia (low levels of red blood cells)

- Thrombocytopenia (low levels of platelets, which increases the risk of bleeding)
- Increased levels of aspartate aminotransferase (AST; a liver enzyme which indicates liver dysfunction)
- Hypogammaglobulinemia (low levels of immunoglobulins [IgG] and antibodies, which increases the risk of infection)

Changes in blood levels are often seen initially and throughout treatment. However, for some patients, changes in blood levels and cell counts can be long-lasting or recur after they are resolved. Your doctor will monitor your blood levels closely throughout treatment and manage accordingly. You may also be advised to take precautions to minimise the risk of falling (due to increased risk of bleeding) and infection (due to low levels of white blood cells/immune cells).

Cilta-cel can cause severe and life-threatening side effects in some patients. The most common, serious side effects observed include:

- Cytokine release syndrome (CRS) (a mild to life-threatening systemic inflammatory response experienced due to the release of cytokines from CAR T-cells)
- Thrombocytopenia (low levels of platelets)
- Febrile neutropenia (low levels of a type of white blood cell known as neutrophils along with a fever)
- Sepsis (an infection in the bloodstream)
- Immune effector cell-associated neurotoxicity syndrome (ICANS)<sup>1</sup>

Of note, there were 21 deaths during the study, six that were considered treatment-related<sup>2</sup>.

Side effects of CAR T-cell therapy often occur during or shortly after treatment, however some side effects may have a delayed onset, or may last longer. While some side effects may be managed with observation and supportive care, others require specific treatments (e.g., CRS is treated with steroids or a monoclonal antibody called tocilizumab). For more detailed information on the side effects related to CAR T-cell therapy, including when during treatment they usually occur, and how they are managed, please, explore our [educational resources on CAR-T therapy](#).



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MPE is a network of European myeloma patient organisations. It supports national patient organisations to improve treatment and access for patients in their countries and helps inform and raise awareness on a European level through its educational programmes. Please note, this information does not replace the information provided by your doctor. If there is anything that is not clear to you, please always ask your clinical team.

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