Myeloma Patients Europe

FACTSHEET

MYELOMA PATIENTS EUROPE

Thalidomide®

www.mpeurope.org

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FACTSHEET

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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 relevant topics related to the disease.

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

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?
- Who should not receive the treatment?
- How and when is the treatment given?

Access the following factsheets on:

- Amyloidosis
- Belantamab mafodotin
- Bortezomib
- Carfilzomib
- Daratumumab
- Elotuzumab
- Ixazomib
- Lenalidomide
- Panobinostat
- Pomalidomide
- Thalidomide
- Stem cell transplant

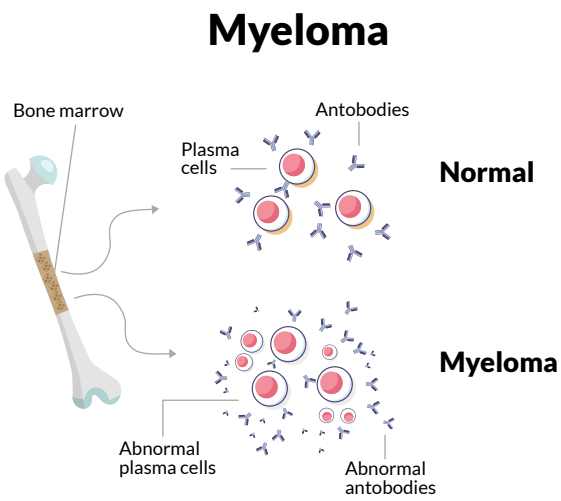
Myeloma treatment is constantly evolving and the factsheets will be updated regularly to reflect the latest developments.

What is myeloma?

Myeloma is a rare cancer of the bone marrow. It is due to the formation of abnormal plasma cells, also called myeloma cells, which divide uncontrollably. Usually, plasma cells help the body to fight infections by making antibodies that recognise and attack germs. 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 require treatment; if the disease responds to therapy, there could be periods of time where symptoms subside and may not require any treatment. This cycle of remission and recurrence (relapse) often occurs several times. Many patients, particularly in relapse setting, will be on treatment for a long period of time to ensure that their myeloma is kept at bay.

Treatment may involve taking a combination of drugs that have been found to be more effective than single drugs. Myeloma generally cannot be cured, but survival rates are increasing in myeloma, due to the availability of new treatment and many patients are able to enjoy a good quality of life. A number of other new treatments have recently been approved or are under consideration for use following relapse, or for refractory myeloma.



What is thalidomide (Thalidomide®)?

According to the European Medicines Agency (EMA), thalidomide is approved in combination with melphalan and prednisone (VMp) in patients with newly diagnosed myeloma that are not candidates for autologous stem cell transplantation. It is used in patients who are older than 65 years or in younger patients if they cannot be treated with high-dose chemotherapy or undergo stem cell transplant.

This combination is one of the standards of care for stem cell transplant-ineligible patients.

Other common and EMA approved combinations are:

- Cyclophosphamide, thalidomide and dexamethasone prior to stem cell transplant
- Bortezomib (Velcade), thalidomide, and dexamethasone (VTd) prior to stem cell transplant
- Other thalidomide based combinations in multiple relapsed setting / heavily pre-treated population

Thalidomide is a medicine that can modulate the activity of the immune system (immunomodulation). It blocks the development of cancer cells and stimulates the body's defence mechanisms. It is from a class of drugs known as immunomodulatory agents, which include similar drugs such as lenalidomide and pomalidomide.

As myeloma is considered a 'rare disease' because of the relatively small number of patients with this disease, thalidomide was granted 'orphan drug designation' by the European Commission* because it is used in a rare disease. An orphan drug designation is a status assigned to medicines developed for rare disease conditions that affect fewer than 5 patients per 10,000 inhabitants in the EU.

How does thalidomide work?

Thalidomide and other immunomodulatory drugs — lenalidomide and pomalidomide — are a group of medicines used in the treatment of myeloma. They are thought to work by blocking the development of cancer cells, and by stimulating some of the specialised cells of the immune system to attack cancer cells. This can help to slow down the progression of myeloma.

Thalidomide modulates the immune system in several ways, of which the best known are:

- It reduces the production by immune cells of a substance (a cytokine) called tumour necrosis factor (TNF-α) and of other pro-inflammatory cytokines, which can promote the growth of cancer cells.
- It reduces a process called angiogenesis, consisting of the generation of new blood vessels, which promotes the growth of tumours.
- It induces the death of myeloma cells.

Who should not receive thalidomide?

Thalidomide is a powerful teratogen, inducing high frequency of severe and life-threatening birth defects. It must never be used in pregnant women or in women who may become pregnant.

What is the Pregnancy Prevention Programme?

Thalidomide must be prescribed and dispensed according to a special Pregnancy Prevention Programme for male and female patients put in place to prevent the exposure of unborn children to the medicine.

Women of childbearing potential must use one effective method of contraception for at least four weeks before start of the treatment, during treatment, and until at least four weeks after thalidomide treatment and even in case of dose interruption unless the patient commits to absolute and continuous abstinence confirmed on a monthly basis. Because of the increased risk of venous thromboembolism in patients with multiple myeloma combined oral contraceptive pills are not recommended.

As thalidomide is found in semen, as a precaution, all male patients taking thalidomide need to use a condom if engaged in sexual activity with a pregnant woman or a woman of childbearing potential not using effective contraception during treatment, during dose interruption and for at least seven days following discontinuation of treatment.

Side effects of thalidomide

Thalidomide may cause fatigue (very common, affecting more than 1 in 10 patients), dizziness (very common), somnolence (very common) and blurred vision (common, affecting between 1 in 10 and 1 in 100 patients). Patients should not drive cars, use machines or perform hazardous tasks while being treated with thalidomide if they feel tired, dizzy, sleepy or have blurred vision. The most common side effects with thalidomide in combination with melphalan and prednisone (seen in more than 1 patient in 10)[1] are:

- Neutropenia (low levels of neutrophils, a type of white blood cell which fights infections)
- Leucopenia (a type of white blood cell which fights infections)
- Anaemia (low red blood cell counts)
- Lymphopenia (low levels of lymphocytes, a type of white blood cell which fights infections)
- Thrombocytopenia (low levels of platelets in the blood; platelets are used by the body to stop bleeding)
- Peripheral neuropathy (nerve damage causing tingling, pain and numbness in the hands and feet)
- Tremor (shaking)
- Dizziness / Paraethesia (unusual sensations like pins and needles)
- Dysaesthesia (reduced sense of touch)
- Somnolence (drowsiness)
- Sleepiness
- Constipation
- Peripheral oedema (swelling, usually in the legs)

Constipation can be often controlled by a combination of generous fluid intake, stool softeners, and laxatives.

Dry skin and itching are frequently noted. They can be prevented by using non-alcohol-based lubricants and by avoiding hot baths. Occasionally, a true skin rash occurs. This requires a temporary cessation of thalidomide, with resumption at a lower dose.

How and when is thalidomide given?

Thalidomide should be taken as a single dose at bedtime, to reduce the impact of drowsiness.

The recommended dose of thalidomide is four capsules a day (i.e., 200 mg, because each capsule contains 50 mg), taken at the same time, preferably at bedtime. In patients over 75 years of age a starting dose of two capsules (100 mg) a day is recommended. For some patients, your haematologist may choose to start at a lower dose and increase gradually to the recommended dose to reduce the risk of side effects (this approach may vary per clinician).

Thalidomide can be used for a maximum of 12 treatment cycles, with each cycle lasting 6 weeks (42 days). The doctor may delay, reduce or stop doses if the patient experiences certain side effects or is having difficulty tolerating the treatment.

Patients also receive an anticoagulant (a medicine to prevent the formation of blood clots) for at least the first five months of treatment.

References

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3. Weber D. Thalidomide and its derivatives: new promise for multiple myeloma. Cancer Control. 2003;10(5):375–383.

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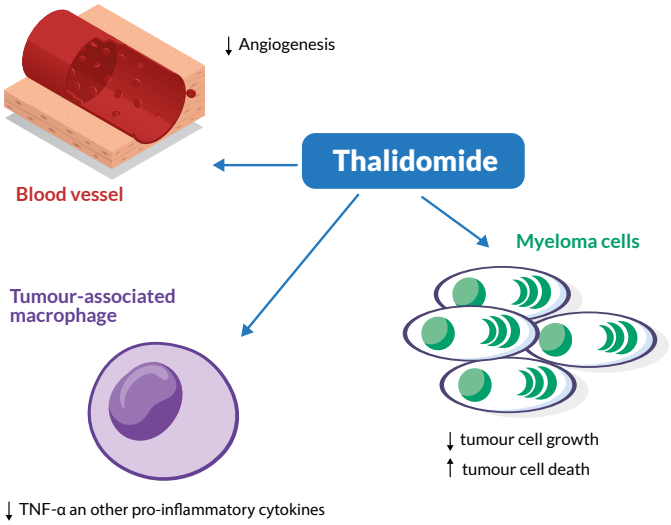
* Thalidomide was withdrawn from the Community Register of designated orphan medicinal products in April 2018 at the end of the 10-year period of market exclusivity.



(**Figure for thalidomide fact-sheet** - Adapted from "Guillerey C, Nakamura K, Vuckovic S, Hill GR, Smyth MJ. Immune responses in multiple myeloma: role of the natural immune surveillance and potential of immunotherapies. Cell Mol Life Sci. 2016;73(8):1569–89.")

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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