

Plain English Summary

Eltrombopag for treating immune thrombocytopenia and severe aplastic anaemia

What does the guidance say?

Eltrombopag is recommended for listing on the Medication Assistance Fund (MAF) for government subsidy for patients with immune thrombocytopenia or severe aplastic anaemia that has not improved after taking other treatments.

What is immune thrombocytopenia?

Immune thrombocytopenia (ITP) is a bleeding disorder caused by abnormally low levels of platelets – the cells in the blood that help blood clot. Symptoms of ITP can include bruising, small bleeds into the skin that may look like a rash (usually on the lower legs), bleeding from the gums or nose, blood in urine or stools, and unusually heavy menstrual flow.

When symptoms do not improve with treatment, this is called refractory ITP.

What is severe aplastic anaemia?

Aplastic anaemia is a rare condition which occurs when your body stops producing enough new blood cells. It can occur suddenly, or it can develop slowly and worsen over time. Symptoms of aplastic anaemia can be mild or severe and include tiring easily, dizziness, pale skin, shortness of breath, rapid or irregular heart rate, bruising, higher risk of infection and bleeding from the gums.

Some cases of severe aplastic anemia are refractory, which means symptoms do not improve with treatment.

What is eltrombopag?

Eltrombopag belongs to a group of medicines called thrombopoietin (TPO) receptor agonists which can make cells in the bone marrow produce more platelets and reduce the risk of bleeding.

It is taken orally each day. Your doctor will tell you how much you need to take and how long you need to take it for.

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Who can have eltrombopag?

Patients with immune thrombocytopenia that has lasted for at least 6 months from diagnosis can have eltrombopag if they cannot tolerate other treatments or their symptoms have worsened despite taking other treatments (such as corticosteroids or immunosuppressants).

Patients with severe aplastic anaemia can have eltrombopag if their symptoms have worsened despite taking immunosuppressive therapy.

Your doctor can advise if eltrombopag is a suitable treatment for you.

Why was eltrombopag recommended for MAF?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Eltrombopag was recommended because its benefit in improving platelet levels and blood counts for certain patients with refractory immune thrombocytopenia and refractory severe aplastic anaemia justifies its cost.

Eltrombopag was not recommended for patients with severe aplastic anaemia who have not had immunosuppressive therapy before because its clinical benefits do not justify its cost.

What does listing on MAF mean for me?

The MAF helps people pay for expensive treatments that are clinically necessary. If your doctor prescribes eltrombopag for you, and you meet the MAF criteria, your treatment cost will be subsidised by 40% to 75%.

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