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Wayrilz (*rilzabrutinib*)

An overview of Wayrilz and why it is authorised in the EU

What is Wayrilz and what is it used for?

Wayrilz is a medicine used to treat immune thrombocytopenia, a disease in which the patient's immune system destroys platelets (components in the blood that help it to clot).It is used in adults for whom treatment with other medicines has not worked.

Immune thrombocytopenia is rare, and Wayrilz was designated an 'orphan medicine' (a medicine used in rare diseases) on 4 June 2020. Further information on the orphan designation can be found on the EMA website.

Wayrilz contains the active substance rilzabrutinib.

How is Wayrilz used?

The medicine can only be obtained with a prescription and treatment should be started and supervised by a doctor who has experience in treating blood diseases.

Wayrilz is available as tablets to be taken by mouth twice a day. Treatment should be stopped after 12 weeks if the platelet levels do not increase enough to prevent bleeding.

For more information about using Wayrilz, see the package leaflet or contact your doctor or pharmacist.

How does Wayrilz work?

The active substance in Wayrilz, rilzabrutinib, binds to and blocks the activity of an enzyme (protein) called Bruton's tyrosine kinase (Btk). This enzyme is involved in activating parts of the immune system. By blocking Btk's activity, rilzabrutinib reduces the immune system's destruction of platelets. This helps increase the number of healthy platelets in the body, thereby reducing the risk of excessive bleeding.

What benefits of Wayrilz have been shown in studies?

Wayrilz was found to be effective in a main study involving 202 adults with immune thrombocytopenia in whom previous treatments had not worked. Patients were treated with either Wayrilz or placebo (a

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dummy treatment). After 24 weeks of treatment, around 23% of those who received Wayrilz (31 out of 133) had achieved a stable platelet count at a level considered adequate to prevent excessive bleeding, compared with none (0%) of those given placebo (0 out of 69).

What are the risks associated with Wayrilz?

For the full list of side effects and restrictions with Wayrilz, see the package leaflet.

The most common side effects with Wayrilz (which may affect more than 1 in 10 people) include diarrhoea, nausea, headache, abdominal (belly) pain, COVID-19, nasopharyngitis (inflammation of the nose and throat) and arthralgia (joint pain).

Why is Wayrilz authorised in the EU?

Treatment for immune thrombocytopenia mainly focuses on preventing bleeding by increasing blood platelet levels. Wayrilz has been shown to increase platelet levels sufficiently to prevent excessive bleeding in some adults with immune thrombocytopenia who have had more than one prior treatment. However, the proportion of patients with a stable response is limited. The product information therefore includes guidance not to continue treatment beyond 12 weeks if the platelet levels have not increased sufficiently. The safety profile of Wayrilz is considered acceptable, with gastrointestinal side effects (affecting the stomach and intestines) being the most common.

The European Medicines Agency therefore decided that Wayrilz's benefits are greater than its risks and that it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Wayrilz?

The company that markets Wayrilz will provide a patient card to remind patients that the medicine should not be used during pregnancy and of the need to use effective birth control during and for one month after treatment.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Wayrilz have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Wayrilz are continuously monitored. Suspected side effects reported with Wayrilz are carefully evaluated and any necessary action taken to protect patients.

Other information about Wayrilz

Wayrilz received a marketing authorisation valid throughout the EU on 22 December 2025.

Further information on Wayrilz can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/wayrilz.

This overview was last updated in 12-2025.