EMA/90810/2025
EMEA/H/C/006105

Rytelo (*imetelstat*)

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

What is Rytelo and what is it used for?

Rytelo is a medicine used to treat anaemia (low levels of red blood cells) in adults with myelodysplastic syndromes, a group of conditions where the bone marrow produces abnormal blood cells and too few healthy ones.

Rytelo is used in patients whose condition does not have an isolated deletion 5q cytogenetic (non-del 5q) abnormality, and who need regular blood transfusions and have a very low to intermediate risk of their condition developing into acute myeloid leukaemia (a blood cancer). It is used in patients for whom erythropoietin (a hormone that stimulates the production of red blood cells) does not work well enough or who cannot be treated with erythropoietin.

Myelodysplastic syndromes are rare, and Rytelo was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 July 2020. Further information on the orphan designation can be found on the EMA website.

Rytelo contains the active substance imetelstat.

How is Rytelo used?

Rytelo is given by infusion (drip) into a vein, usually over a period of around two hours; it is given once every four weeks. The recommended dose depends on the patient's weight and can be adjusted in case of side effects. At least 30 minutes before each infusion, patients should be given medicines to prevent or reduce potential side effects of the infusion.

A complete blood cell count and liver function tests should be performed before each dose. After the first two doses, weekly blood cell counts are recommended. Before starting treatment, women able to have children should do a pregnancy test.

Treatment should be stopped if the need for blood transfusions is not reduced after 24 weeks (six doses) or if the side effects become unacceptable.

Rytelo can only be obtained with a prescription. Treatment should be given and monitored under the supervision of healthcare professionals experienced in the treatment of blood diseases.

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

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How does Rytelo work?

The active substance in Rytelo, imetelstat, blocks the activity of an enzyme called telomerase that helps cells grow and divide. By blocking telomerase, the medicine reduces the growth of abnormal blood cells and promotes their death.

What benefits of Rytelo have been shown in studies?

A main study involved 178 adults with myelodysplastic syndromes requiring regular blood transfusions; patients received either Rytelo or placebo (a dummy treatment) in addition to supportive care.

The study showed that 36 out of 118 patients (30.5%) given Rytelo did not need a blood transfusion for at least 8 weeks, compared with 6 out of 60 (10%) patients receiving placebo.

What are the risks associated with Rytelo?

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

The most common side effects with Rytelo (which may affect more than 1 in 10 people) include thrombocytopenia (low levels of blood platelets, components that help the blood to clot), neutropenia (low levels of neutrophils, a type of white blood cell that fights infections), increased levels of liver enzymes (a sign of possible liver problems), tiredness and headache.

Some side effects can be serious. The most common serious side effects (which may affect up to 1 in 10 people) include sepsis (when bacteria and their toxins circulate in the blood leading to organ damage), urinary tract infection (infection of the parts of the body that collect and pass out urine), atrial fibrillation (irregular and uncoordinated contractions of the upper chambers of the heart), oesophageal varices haemorrhage (bleeding from swollen veins in the lining of the oesophagus, the tube that leads from the mouth to the stomach), syncope (fainting), and thrombocytopenia.

Why is Rytelo authorised in the EU?

Treatment with frequent blood transfusions can lead to accumulation of iron in the body, which can damage organs. Rytelo can reduce the need for blood transfusions in patients with myelodysplastic syndromes, while its side effects are considered manageable with the additional measures in place such as blood cell count and liver function monitoring.

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

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

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

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

Other information about Rytelo

Rytelo received a marketing authorisation valid throughout the EU on 07 March 2025.

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

This overview was last updated in 03-2025.