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Jakavi (*ruxolitinib*)

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

What is Jakavi and what is it used for?

Jakavi is a medicine used to treat the following conditions:

- splenomegaly (enlarged spleen) or other disease-related symptoms such as fever, night sweats, bone pain and weight loss in adults who have myelofibrosis. Myelofibrosis is a disease in which the bone marrow becomes very dense and rigid and produces abnormal, immature blood cells.
- polycythaemia vera in adults in whom the medicine hydroxycarbamide (also known as hydroxyurea) does not work or causes unacceptable side effects. In polycythaemia vera, too many red blood cells are produced, which can cause reduced blood flow to the organs due to 'thickening' of the blood and occasionally the formation of blood clots.
- acute or chronic graft-versus-host disease (when transplanted cells attack the body) in people aged 12 and older who have had a transplantation and for whom corticosteroids or other systemic therapies (treatments given by mouth or injection) did not work well enough.

Jakavi contains the active substance ruxolitinib.

How is Jakavi used?

The medicine can only be obtained with a prescription. Treatment with Jakavi should only be started by a doctor who is experienced in treating patients with cancer medicines.

Jakavi is available as tablets taken twice a day. The recommended dose depends on the condition it is used for.

The dose should be reduced or the treatment should be stopped if certain side effects occur.

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

How does Jakavi work?

The active substance in Jakavi, ruxolitinib, works by blocking a group of enzymes known as Janus kinases (JAKs), which are involved in the production and growth of blood cells. In myelofibrosis and polycythaemia vera, there is too much JAK activity, leading to the abnormal production of blood cells.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

These blood cells migrate to organs, including the spleen, causing the organs to become enlarged. JAKs are also involved in the development and activation of blood cells that play a role in graft-versus-host disease. By blocking JAKs, Jakavi reduces the production of blood cells, thereby reducing the symptoms of the diseases.

What benefits of Jakavi have been shown in studies?

Myelofibrosis

Jakavi was more effective than placebo and the best available treatment for reducing the size of the spleen in two main studies involving 528 patients. In the first study, the target 35% reduction in spleen size after 6 months was achieved in 42% of patients treated with Jakavi (65 out of 155) compared with less than 1% of patients given placebo (1 out of 153). In the second study, the target 35% reduction in spleen size after one year was achieved in 29% of patients treated with Jakavi (41 out of 144) compared with none of the 72 patients receiving the best available treatment, such as cancer medicines, hormones and immunosuppressants.

Polycythaemia vera

Jakavi improved patients' condition in one main study which involved 222 patients in whom hydroxycarbamide did not work or caused unacceptable side effects. Improvement was measured as requiring fewer than one phlebotomy treatment (to remove excess blood from the body) and a reduction in spleen size of at least 35%. In this study, 21% (23 out of 110) of patients given Jakavi had an improvement in their condition after 8 months of treatment, compared with 1% (1 out of 112) of patients given the best available treatment.

Graft-versus-host disease

Jakavi was effective at reducing the symptoms of both acute and chronic graft-versus-host disease in 2 main studies.

The first study involved 309 patients with acute graft-versus-host disease after allogeneic stem cell transplantation (using stem cells from a donor) in whom corticosteroid therapy did not work. It looked at the proportion of patients who had reduced symptoms (partial response) or no sign of symptoms (complete response) after 4 weeks of treatment with Jakavi or the best available treatment for their disease. In this study 62% of patients (96 out of 154) who received Jakavi had either a complete or a partial response to treatment compared with 39% of patients (61 out of 155) who received another therapy.

The second study involved 329 patients with chronic graft-versus-host disease after allogeneic stem cell transplantation in whom corticosteroid therapy did not work. In this study, after 24 weeks of treatment 50% of patients who received Jakavi (82 out of 165) had either a complete or partial response compared with 26% (42 out of 164) of patients who received the best available treatment for their disease.

What is the risk associated with Jakavi?

In myelofibrosis, the most common side effects with Jakavi (which may affect more than 1 in 10 people) include thrombocytopenia (low blood platelet counts), anaemia (low red blood cell counts), neutropenia (low levels of neutrophils), bleeding, bruising, hypertriglyceridaemia (high blood fat levels), dizziness, and raised liver enzyme levels.

In polycythaemia vera, the most common side effects with Jakavi (which may affect more than 1 in 10 people) include thrombocytopenia, anaemia, weight gain, headache, dizziness, hypercholesterolaemia (high blood cholesterol levels) and raised liver enzyme levels.

In acute graft-versus-host disease, the most common side effects with Jakavi (which may affect more than 1 in 10 people) include thrombocytopenia, anaemia, neutropenia, cytomegalovirus infection, sepsis (when bacteria and their toxins circulate in the blood leading to organ damage), urinary tract (structures that carry urine) infections, hypercholesterolaemia and raised liver enzyme levels.

In chronic graft-versus-host disease, the most common side effects with Jakavi (which may affect more than 1 in 10 people) include thrombocytopenia, anaemia, neutropenia, hypertension, headache, urinary tract infections, hypercholesterolaemia and raised liver enzyme levels.

Women who are pregnant or breastfeeding must not take Jakavi. For the full list of side effects and restrictions of Jakavi, see the package leaflet.

Why has Jakavi been authorised in the EU?

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

In myelofibrosis, the reduction in spleen size and in symptoms in patients taking Jakavi is clinically important and patients' quality of life is improved. In polycythaemia vera the Agency considered that Jakavi is of benefit to patients when treatment with hydroxycarbamide does not work or causes unacceptable side effects. In the treatment of graft-versus-host disease, Jakavi has been shown to reduce the symptoms.

In terms of safety, the Agency considered that Jakavi's side effects can be appropriately managed.

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

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

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

Other information about Jakavi

Jakavi was granted a marketing authorisation valid throughout the EU on 23 August 2012.

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

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