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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 graft-versus-host disease (GvHD, when donor cells attack the body shortly after a transplant) in adults and children aged 28 days and older for whom corticosteroids or other systemic therapies (treatments given by mouth or injection) did not work well enough;
- chronic graft-versus-host-disease (which usually develops later than acute GvHD, within several weeks to months after a transplant) in adults and children aged 6 months and older for whom corticosteroids or other systemic therapies did not work well enough.

Jakavi contains the active substance ruxolitinib.

## How is Jakavi used?

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

Jakavi is available as tablets and a solution taken by mouth, which are each taken twice daily. 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.

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## 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. These blood cells can move to organs, including the spleen, causing the organs to become enlarged. JAKs are also involved in the development and activation of cells of the immune system (the body's natural defences) that play a role in GvHD. By blocking JAKs, ruxolitinib helps to reduce inflammation, thereby reducing the symptoms of acute and chronic GvHD.

## What benefits of Jakavi have been shown in studies?

### Myelofibrosis

Jakavi was more effective than placebo (dummy treatment) and the best available treatment for reducing the size of the spleen in two main studies involving 528 adult 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 adult 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 adults and adolescents aged 12 years and older in 2 main studies.

The first study involved 309 adults and adolescents aged 12 years and older, with acute GvHD due to an allogeneic stem cell transplant (using stem cells from a donor). Corticosteroid therapy for acute GvHD did not work in these patients. The study 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. Results showed that 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 adults and adolescents aged 12 year and older, with chronic GvHD due to an allogeneic stem cell transplant. Corticosteroid therapy for chronic GvHD did not work in these patients. 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.

Data on how Jakavi behaves in the body showed that, when the medicine is given to children under 12 years at the recommended doses for the treatment of acute and chronic GvHD, its blood levels are similar to those seen in adults.

## What is the risk associated with Jakavi?

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

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 GvHD, the most common side effects with Jakavi in adults and adolescents (which may affect more than 1 in 10 people) include thrombocytopenia, anaemia, neutropenia and increased levels of the liver enzymes alanine aminotransferase and aspartate aminotransferase. The most common side effects with Jakavi in adolescents and children (which may affect more than 1 in 10 people) include thrombocytopenia, anaemia, neutropenia, hypercholesterolaemia and increased levels of alanine aminotransferase.

In chronic GvHD, the most common side effects with Jakavi in adults and adolescents (which may affect more than 1 in 10 people) include anaemia, hypercholesterolaemia and increased levels of aspartate aminotransferase. The most common side effects in children and adolescents (which may affect more than 1 in 10 people) include neutropenia, hypercholesterolaemia and increased levels of alanine aminotransferase.

Women who are pregnant or breastfeeding must not take Jakavi.

## 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 adults and adolescents aged from 12 years. Based on how the medicine works, its effectiveness and safety profile for treating acute and chronic GvHD in younger children are expected to be the same as in adults.

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.

This overview was last updated in 12-2024.