EMA/78494/2026
EMEA/H/C/005927

Joenja (*leniolisib*)

A plain-language overview of Joenja and why it is authorised in the EU

What is Joenja and what is it used for?

Joenja is a medicine used to treat activated phosphoinositide 3-kinase delta syndrome (APDS) in adults and adolescents from 12 years of age and weighing 45 kg or more.

APDS is a rare, inherited disease in which the immune system (the body's natural defences) does not work properly, making patients more susceptible to bacterial and viral infections. The condition can also lead to autoimmune disorders and lymphoma (cancer of lymphocytes, a type of white blood cell).

APDS is rare, and Joenja was designated an 'orphan medicine' (a medicine used in rare diseases) on 19 October 2020. Further information on the orphan designation can be found on the EMA website.

Joenja contains the active substance leniolisib.

How is Joenja used?

The medicine can only be obtained with a prescription. Treatment must be started by a doctor with experience in the management of primary immune deficiencies (when the immune system does not function properly).

Joenja is available as tablets to be taken by mouth twice a day, approximately 12 hours apart. Treatment should continue for as long as the patient benefits from it, or until side effects become unacceptable.

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

How does Joenja work?

People with APDS have mutations (changes) in the genes that control the production of a protein called phosphoinositide 3-kinase delta. This protein is essential for the development and function of lymphocytes (B and T cells), which play a key role in the immune system. The mutations make the protein overactive, interfering with the normal development and function of lymphocytes. This leads to increased levels of immature B cells that build up in the lymph nodes (lymphadenopathy) and organs

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

© European Medicines Agency, 2026. Reproduction is authorised provided the source is acknowledged.

such as the spleen, liver or lungs, as well as reduced levels of naïve B cells (newly formed mature B cells involved in the initial immune response), affecting the patient's ability to fight infections.

The active substance in Joenja, leniolisib, attaches to phosphoinositide 3-kinase delta and reduces its activity, leading to the normal development and function of B and T cells.

What benefits of Joenja have been shown in studies?

One main study involved 31 people from 12 years of age with APDS, who received either Joenja or placebo (a dummy treatment) along with standard treatment to manage symptoms of the condition. The study looked at the change in the level of lymphadenopathy and in the proportion of naïve B cells after 12 weeks of treatment.

People taking Joenja had a greater reduction in lymphadenopathy than those taking placebo, indicating a reduction in lymphoproliferation (abnormal production of lymphocytes). In addition, there was a greater increase in the proportion of naïve B cells in people taking Joenja than in those given placebo, indicating that B cell development may have normalised.

Studies carried out with Joenja are described in more detail in the medicine's assessment report.

What are the side effects and restrictions with Joenja?

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

The most common side effects with Joenja (which may affect more than 1 in 10 people) include decreased levels of neutrophils (a type of white blood cell), headache, vomiting, increased weight and alopecia (hair loss).

Why is Joenja authorised in the EU?

Joenja was shown to be effective at reducing lymphadenopathy and increasing the number of naïve B cells in people with APDS, which is expected to reduce the risk of infection and other complications associated with the condition. The safety profile was considered acceptable, with generally manageable side effects. The European Medicines Agency therefore decided that Joenja's benefits are greater than its risks and that it can be authorised for use in the EU.

Joenja has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Joenja due to the rarity of the disease. The company must provide further data on Joenja. It must submit the results from a registry-based study on the long-term safety and effectiveness of Joenja, and must provide yearly updates on any new information concerning the medicine's safety and effectiveness. Every year, the Agency will review any new information that becomes available.

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

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

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

Other information about Joenja

Joenja received a marketing authorisation under exceptional circumstances valid throughout the EU on 21 May 2026.

Further information on Joenja, including the package leaflet and assessment report, can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/joenja.

For information about the availability of this medicine in your country, contact your national competent authority.

This overview was last updated in 05-2026.