



26 March 2026  
EMA/CHMP/28350/2026  
Committee for Medicinal Products for Human Use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

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### Joenja leniolisib

On 26 March 2026, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances<sup>2</sup> for the medicinal product Joenja<sup>3</sup>, intended for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS) in adults and adolescents 12 years of age and older and weighing 45 kg or more.

The applicant for this medicinal product is Pharming Technologies B.V.

Joenja will be available as 70 mg film-coated tablets. The active substance of Joenja is leniolisib, an immunostimulant (ATC code: L03AX22). APDS is a complex primary immunodeficiency disease caused by a mutation in either the *PIK3CD* or *PIK3R1* gene. Leniolisib is a PI3K $\delta$  inhibitor targeting the catalytic p110 $\delta$  subunit of PI3K $\delta$ , thereby specifically inhibiting the hyperactivated PI3K pathway, which underlies APDS. As a result, deficiencies and dysregulation of B and T cell populations normalise.

The benefits of Joenja are an improvement in lymphadenopathy (swollen/enlargement of lymph nodes), and immunophenotype normalization (increase in percentage of naïve B-cells out of total B-cells), as observed in a double-blind, randomised, placebo-controlled study in 31 patients with APDS. Improvements were also observed on spleen parameters (reduced mean sum of product diameter volume of index lesion and mean bi-dimensional size and volume of spleen), but an effect on reducing infections frequency could not be shown.

The most common side effects are headache, vomiting, alopecia, weight increased and neutrophil count decreased.

The full indication is:

Joenja is indicated for the treatment of activated phosphoinositide 3-kinase delta syndrome

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<sup>1</sup> Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

<sup>2</sup> In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.

<sup>3</sup> This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained



(APDS) in adults and adolescents 12 years of age and older and weighing 45 kg or more.

Treatment with Joenja should be initiated by physicians experienced in the management of primary immune deficiencies.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.