13 November 2025
EMA/343921/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Waskyra

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On 13 November 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Waskyra[2], intended for the treatment Wiskott-Aldrich Syndrome (WAS). As Waskyra is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies.

The applicant for this medicinal product is Fondazione Telethon ETS.

Waskyra will be available as a 2–10 x 10^6 cells/ml dispersion for infusion. The active substance of Waskyra consists of genetically modified autologous CD34+ haematopoietic stem cell enriched population transduced *ex vivo* with a lentiviral vector encoding the human WAS gene. Waskyra is a gene therapy (ATC code: not yet assigned) that inserts the WAS corrected gene into the cell's genome, making the genetically modified cells capable of expressing the functional WAS protein. The genetically modified cells engraft and repopulate the haematopoietic compartment. They differentiate and produce biologically active lymphoid and myeloid progenitors whose progeny express WAS protein.

The benefits of Waskyra are a decrease in the annualised rate of severe infections from 2.00 per person-year in the 12 months of observation before Waskyra infusion to 0.15 in the 1 to 2 years after infusion, and 0.12 in the 2 to 3 years after infusion, as shown in a single arm phase 3 trial. The annualised rate of moderate and severe bleeding events also decreased from 2.00 events per person-year in the 12 months before Waskyra infusion to 0.16 in the 2 to 3 years after infusion. Overall, 96% of patients survived (95% CI: 82–99%) following treatment with Waskyra.

The most common side effects with Waskyra were attributed to pre-treatment procedures (including mobilisation and leukapheresis), the conditioning regimen and administration site conditions (with device-related infections and catheter site haemorrhages reported very commonly).

The full indication is:

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] 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

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Waskyra is indicated for the treatment of patients aged 6 months and older with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene for whom haematopoietic stem cell (HSC) transplantation is appropriate and no suitable human leukocyte antigen (HLA)-matched related haematopoietic stem cell donor is available.

Waskyra must be administered in a qualified treatment centre by a physician with experience in haematopoietic stem cell transplantation (HSCT) and trained for administration and management of patients treated with the medicinal product.

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.