

ALASKA MEDICAID
Prior Authorization Criteria

Waskyra
(etuvetidigene-autoemcel)

FDA INDICATIONS AND USAGE¹

Waskyra is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of pediatric patients aged 6 months and older and adults with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched stem cell donor is available.

APPROVAL CRITERIA^{1,2,3,4}

1. Patient meets FDA labeled age **AND**;
2. Prescribed by or in consultation with a Wiskott-Aldrich Syndrome specialist (e.g. hematologist, immunologist, etc.) **AND**;
3. Patient has the diagnosis of Wiskott-Aldrich Syndrome (WAS), confirmed by genetic mutation identified by genetic testing with severe phenotype WAS mutation **AND**;
4. Patient exhibits one or both of the following:
 - a. Absent or significantly diminished Wiskott-Aldrich Syndrome protein (WASP) expression
 - b. Clinically severe phenotype as evidenced by Zhu or Ochs score ≥ 3 **AND**;
5. Patient does not have an identified medically eligible human leukocyte antigen (HLA)-matched stem cell transplant donor **AND**;
6. Patient is confirmed negative for HIV-infection and Hepatitis C **AND**;
7. Patient will have mobilization of stem cells using granulocyte-colony stimulating factor (G-CSF) and plerixafor according to established protocols.

DENIAL CRITERIA¹

1. Failure to meet approval criteria **OR**;
2. Patient has previously received hematopoietic stem cell-based gene therapy **OR**;
3. Patient has received hematopoietic stem cell-based transplant within the previous 6 months, or prior with evidence of residual donor cells present **OR**;
4. Patient has a clinical contraindication to any aspect of the mobilization or conditioning regimen.

CAUTIONS¹

- Patients should be monitored for signs and symptoms of failed engraftment.
- Due to risk of severe cytopenias following Waskyra, patients should be monitored for cytopenias for at least 8 weeks following treatment.
- Treatment with Waskyra may affect the accuracy of HIV PCR-based screening.
- Following Waskyra treatment, there is a lifelong risk of lentiviral vector (LVV)-mediated insertional oncogenesis and secondary malignancy.
- Serious infections have occurred with Waskyra administration and the accompanying administration of rituximab and conditioning regimen.

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- Patients treated with Waskyra should not donate blood, organs, cells, or tissues for transplantation at any time in the future.

DURATION OF APPROVAL

- Initial Approval: up to 6 months
- Reauthorization will not be approved

QUANTITY LIMIT

- One infusion per lifetime
- HCPCS: J3386

REFERENCES / FOOTNOTES:

1. Waskyra (etuvetidigene autotemcel) [prescribing information]. Rome, Italy: Fondazione Telethon ETS; December 2025.
2. NCT01515462. Gene therapy for Wiskott-Aldrich Syndrome (TIGET-WAS). Updated April 4, 2025.
3. NCT03837483. A clinical study to evaluate the use of a cryopreserved formulation of OTL-103 in subjects with Wiskott-Aldrich Syndrome. Updated September 29, 2025.
4. Ochs HD, Filipovich AH, Veys P, et.al. Wiskott-Aldrich Syndrome: Diagnosis, Clinical and Laboratory Manifestations, and Treatment. *Biology of Blood and Marrow Transplantation*, 2009;15: 84-90.