CareSource Rx Innovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Waskyra (etuvetidigene autotemcel)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

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 related stem cell donor is available.
WAS is a rare x-linked disorder affecting the immune system due to a mutation in the WAS gene. It is characterized by immune deficiency, thrombocytopenia and eczema. Treatment consists of supportive care and, if a compatible donor is available, a hematopoietic stem cell transplantation.

Waskyra (etuvetidigene autotemcel) will be considered for coverage when the following criteria are met:

Wiskott-Aldrich Syndrome (WAS)

For **initial** authorization:
1. Member is male; AND
2. Member is at least 6 months of age; AND
3. Medication must be prescribed by or in consultation with an immunologist, hematologist, bone marrow transplant specialist or provider experienced in treating WAS; AND
4. Member has a diagnosis of WAS confirmed by genetic testing showing a WAS mutation and **ONE** of the following:
   a) Severe WAS gene mutation
   b) Absent WASP expression
   c) Several clinical score (Zhu score ≥ 3); AND
5. Provider attests hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available; AND
6. Member does **NOT** have any of the following:
   a) Previous treatment with HSCT within 6 months prior to screening;
   b) HSCT with evidence of residual donor cell;
   c) Prior use of hematopoietic stem cell gene therapy; AND
7. Member's weight is provided for dose calculation.
8. **Dosage allowed/Quantity limit:** The minimum recommended dose is 7 x 10^6 CD34+ cells per kg.

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:
1. Waskyra will not be reauthorized.

CareSource considers Waskyra (etuvetidigene autotemcel) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
01/02/2026	New policy for Waskyra created.

References:

1. Waskyra [prescribing information]. Fondazione Telethon ETS; 2025.
2. Vallée TC, Albert MH, Pai SY. How I treat Wiskott-Aldrich syndrome. *Blood*. 2025;146(1):41-51. doi:10.1182/blood.2024026288
3. Ferrua F, Cicalese MP, Galimberti S, et al. Lentiviral haemopoietic stem/progenitor cell gene therapy for treatment of Wiskott-Aldrich syndrome: interim results of a non-randomised, open-label, phase 1/2 clinical study. *Lancet Haematol*. 2019;6(5):e239-e253. doi:10.1016/S2352-3026(19)30021-3
4. A Clinical Study to Evaluate the Use of a Cryopreserved Formulation of OTL-103 in Subjects With Wiskott-Aldrich Syndrome. ClinicalTrials.gov identifier: NCT03837483. Updated September 9, 2025. Accessed January 6, 2026. https://clinicaltrials.gov/study/NCT03837483

Effective date: 07/01/2026
Revised date: 01/02/2026