Clinical Policy: Etuvetidigene Autotemcel (Waskyra)

Reference Number: PA.CP.PHAR.735
Effective Date: 02/2026
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Description

Etuvetidigene autotemcel (Waskyra™) is an autologous hematopoietic stem cell-based gene therapy.

FDA Approved Indication(s)

Waskyra is 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.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Waskyra is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Wiskott-Aldrich Syndrome (must meet all)**:**

1. Diagnosis of WAS confirmed by the presence of a WAS genetic mutation and one of the following (a, b, c, or d; see *Appendix D*):
   a. Severe WAS gene mutation;
   b. Absent or markedly reduced WAS protein expression;
   c. Severe WAS clinical phenotype defined as a Zhu, Ochs, or Zhu-Ochs clinical score of 3 or higher;
   d. Clinically significant disease as evidenced by documented classic clinical manifestations of WAS (e.g., microthrombocytopenia with bleeding, recurrent or severe infections, eczema, immune dysfunction or autoimmunity);
2. Prescribed by or in consultation with a medical geneticist, transplant specialist, or specialist with expertise in treating WAS (e.g., hematologist, immunologist);
3. Age ≥ 6 months;
4. Member has no available HLA-matched related stem cell donor;
5. Transplant specialist attestation that member is clinically stable and eligible to undergo myeloablative conditioning and HSCT;
6. If member has previously received allogeneic HSCT, both of the following (a and b):
   a. It has been > 6 months since the transplant;
   b. There is no evidence of residual cells of donor origin;
7. Member has not received prior hematopoietic stem cell gene therapy;
8. Dose is a single infusion containing a minimum of 7 x 10^6 $CD34^+$ cells per kg.

Approval duration: 3 months (one time infusion per lifetime)

B. Other diagnoses/indications
1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Wiskott-Aldrich Syndrome
1. Re-authorization is not permitted as Waskyra is indicated to be dosed one time only.

Approval duration: Not applicable

A. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53
B. X-linked thrombocytopenia (XLT);
C. X-linked neutropenia (XLN).

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration
HLA: human leukocyte antigen
HSCT: hematopoietic stem cell transplantation
WAS: Wiskott-Aldrich syndrome
XLN: X-linked neutropenia
XLT: X-linked thrombocytopenia

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): hypersensitivity to the active substance or any of the excipients; previous treatment with HSCT within 6 months prior to screening or HSCT with evidence of residual donor cells; previous treatment with hematopoietic stem cell gene therapy; contraindications to the mobilization and the conditioning regimen
- Boxed warning(s): none reported

Appendix D: General Information

- Mutations in the WAS gene result in variable clinical phenotypes categorized into 3 major groups: classic (severe) WAS phenotype (approximately 50% of patients); XLT phenotype, a milder form of WAS (nearly all others), and XLN phenotype (more rare).

- Severe WAS gene mutations include nonsense, frameshift caused by deletions or insertions, splice-site mutation, missense, and inversion.
- Zhu-Ochs scoring system
  - The severity of WAS-associated symptoms can be estimated through a scoring system developed by Zhu et al in 1995. The Ochs system is an updated and refined version of the original system proposed by Zhu. They are often used in conjunction and referred to as the Zhu-Ochs system.
    - Zhu et al: generally assigned one point for each clinical feature (thrombocytopenia, infections, eczema)
    - Ochs et al: modified the scale and assigned points or ranges to different severities of symptoms
  - For XLT patients: A score of 0.5 or 1, assigned to patients with intermittent or chronic thrombocytopenia and small platelets, and a score of 2, assigned to patients with additional findings of mild, transient eczema or minor infections, identify XLT patients.
  - For WAS patients:
    - Those with treatment-resistant eczema and recurrent infections despite optimal treatment receive a score of 3 (mild WAS) or 4 (severe WAS).
    - Regardless of the original score, if a patient develops autoimmune disease or malignancy, a score of 5 is attributed. Scores 5A and 5M indicate a score of 5 with autoimmune disease (A) or malignancy (M), respectively.

Clinical Scores	XLT			WAS			
	0.5	1	2	3	4	5A	5M
Thrombocytopenia	+/-	+	+	+	+	+	+
Eczema	-	-	+/-	+	++	++/-	++/-
Immunodeficiency	-	-	+/-	+	++	++/-	++/-
Autoimmunity	-	-	-	-	-	+	-
Malignancy	-	-	-	-	-	-	+

+ indicates present; - indicates absent; +/- indicates present-mild or absent; ++ indicates present-severe; and ++/- indicates present severe or absent

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
WAS	Minimum recommended dose: 7 x 10^6 $CD34^+$ cells/kg IV as a one-time dose	None

VI. Product Availability

Single-dose cell suspension: one to eight infusion bags overall containing a suspension of 2 – 11.4 x 10^6 cells/mL (1.9 – 11.4 x 10^6 $CD34^+$ cells/mL) in cryopreservative solution

VII. References

1. Waskyra Prescribing Information. Rome, Italy: Fondazione Telethon ETS.; December 2025. Available at: https://www.fda.gov/media/190096/download?attachment. Accessed December 16, 2025.
2. Ochs HD and Trasher AJ. The Wiskott-Aldrich syndrome. J Allergy Clin Immunol. 2006 April;117(4):725-738;

3. Buchbinder D, Nugent DJ, Fillipovich AH. Wiskott-Aldrich syndrome: Diagnosis, current management, and emerging treatments. Appl Clin Genet 2014;7:55-56.
4. Bosticardo M, Maragoni F, Aiuti A, Villa A, and Roncarolo MG. Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. *Blood* 2009;133(25):6288-6295.
5. Ferrua F, Pia Cicalese M, Galimberti S, et al. Lentiviral haemopoietic stem/progenitor cell therapy for treatment of Wiskott-Aldrich syndrome: Interim results of a non-randomized, open-label, phase 1/2 clinical study. Lancet Haematol. 2019 Apr 10;6(5):e239-e253.
6. ClinicalTrials.gov. Gene therapy for Wiskott-Aldrich syndrome (TIGET-WAS). Available at: https://clinicaltrials.gov/study/NCT01515462. Assessed December 17, 2025.
7. ClinicalTrials.gov. A clinical study to evaluate the use of a cryopreserved formulation of OTL-103 in subjects with Wiskott-Aldrich syndrome. Available at: https://clinicaltrials.gov/study/ NCT03837483. Assessed December 17, 2025.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date
Policy created	01/2026