

Clinical Policy: Etuvetidigene Autotemcel (Waskyra)

Reference Number: CP.PHAR.735

Effective Date: 12.09.25

Last Review Date: 02.26

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)
[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

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.

All requests reviewed under this policy **require Precision Drug Action Committee (PDAC) Utilization Management Review**. Refer to CC.PHAR.21 for process details.

It is the policy of health plans affiliated with Centene Corporation[®] 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 \geq 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 7×10^6 CD34⁺ cells per kg.
- Approval duration: 3 months (one time infusion per lifetime)**

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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**

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line

of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents;
- 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: 7×10^6 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 10⁶ cells/mL (1.9 – 11.4 x 10⁶ 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	P&T Approval Date
Policy created pre-emptively	06.03.25	08.25
Drug is now FDA approved – criteria updated per FDA labeling: for diagnostic criteria, added option for Ochs or Zhu-Ochs clinical score and documented classic clinical manifestations of WAS; revised minimum age requirement from 1 year to 6 months; removed member is not positive for the presence of HIV type 1 or 2; removed option for those with an HLA-matched donor; removed option for HLA-matched unrelated donor for those age < 5 years; for prior allogeneic HSCT, revised to allow if it has been > 6 months since the transplant and there is no evidence of residual cells of donor origin; updated dose requirement to a minimum single dose; references reviewed and updated.	01.13.26	02.26

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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