Reference number(s)
7335-A

Standard Medicare Part B Management Waskyra

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Waskyra	etuvetidigene autotemcel

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

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 and for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

- Genetic testing results demonstrating a pathogenic variant in the WAS gene.

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- Chart notes, medical records, or lab results documenting either of the following:
    - Flow cytometry results showing level of Wiskott-Aldrich Syndrome Protein (WASP) expression.
    - Zhu clinical score confirming severe clinical phenotype (score greater than or equal to 3, see Appendix A).

Coverage Criteria

Wiskott-Aldrich Syndrome (WAS)[1-4]

Authorization of 3 months for a one-time administration may be granted for treatment of Wiskott-Aldrich Syndrome (WAS) when all of the following criteria are met:

- Member is 6 months of age or older.
- Member has a diagnosis of Wiskott-Aldrich Syndrome (Classic WAS) confirmed by the presence of a pathogenic variant in the WAS gene and either of the following:
    - Absent or truncated Wiskott-Aldrich Syndrome protein (WASP) expression assessed by flow cytometry, or
    - Severe clinical phenotype (Zhu clinical score greater than or equal to 3, see Appendix A).
- Member is an appropriate candidate for hematopoietic stem cell transplant (HSCT), and meets either of the following criteria:
    - Member is 5 years of age or older and has no human leukocyte antigen (HLA)-identical sibling donor, or
    - Member is less than 5 years of age and has no HLA-identical sibling donor, suitable 10/10 HLA matched unrelated donor, or 6/6 HLA matched unrelated cord blood donor.
- Member does not have evidence of residual cells of donor origin if the member has received a prior allogeneic hematopoietic stem cell transplant (allo-HSCT) and has not received allo-HSCT in the past 6 months.
- Member has not received Waskyra or any other gene therapy previously.

Appendix

Appendix A: WAS-Related Disorders: Clinical Scoring System[3,4]

Feature					Score	Phenotype
Thrombocytopenia	**Eczema**	**Immunodeficiency**	**Autoimmune Disorders**	**Malignancy**		

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Absent	Absent	Absent	Absent	Absent	0	XLN / myelodysplasia
Present	Absent	Absent	Absent	Absent	1	XLT
Present	Mild or transient	Infrequent infections	Absent	Absent	2	
Present	Persistent but responsive	Recurrent infections	Absent	Absent	3	Wiskott-Aldrich Syndrome
Present	Severe, not controlled	Severe infections	Absent	Absent	4	
Present	Any	Any	Present	Present	5	XLT/Wiskott-Aldrich syndrome w/autoimmunity &/or malignancy

XLN = X-linked neutropenia; XLT = X-linked thrombocytopenia

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Waskyra.
- The available compendium
  - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
  - Micromedex DrugDex
  - American Hospital Formulary Service- Drug Information (AHFS-DI)
  - Lexi-Drugs
  - Clinical Pharmacology
- 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
- WAS-related Disorders - Gene Reviews
- American Society of Hematology: Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome.

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Waskyra are covered.

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Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for utilization of the Zhu clinical score to aid in Wiskott-Aldrich Syndrome diagnosis can be found in a review published by the American Society of Hematology. The severity of WAS-associated symptoms can be estimated through a scoring system developed by Zhu et al. 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 X-linked thrombocytopenia (XLT) 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.

References

1. Waskyra [package insert]. Rome, Itay: Fondazione Telethon ETS.; December 2025.
2. 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
3. Chandra S, Nagaraj CB, Sun M, et al. WAS-Related Disorders. 2004 Sep 30 [Updated 2024 Aug 15]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1178/ Internal References.
4. Bosticardo M, Marangoni F, Aiuti A, Villa A, Grazia Roncarolo M. Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. Blood. 2009;113(25):6288-6295. doi:10.1182/blood-2008-12-115253

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