| Reference number(s) |
| --- |
| 7334-A |

# Specialty Guideline 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 are considered experimental/investigational and not medically necessary.

## Documentation

Submission of the following information is necessary to initiate the prior authorization review:

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

# Prescriber Specialties

This medication must be prescribed by or in consultation with an immunologist, hematologist, or physician who specializes in the treatment of Wiskott-Aldrich Syndrome (WAS).

# 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 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.
  - 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 is negative for human immunodeficiency virus (HIV) and hepatitis C infection.
- Member does not have acute or chronic stable hepatitis B.
- Member does not have symptomatic herpes zoster that is unresponsive to specific treatment.
- Member does not have evidence of acute tuberculosis.
- Member is not affected by malignant neoplasia (except local skin cancer).
- Member does not have a documented history of hereditary cancer syndrome.
- Member is not affected by cytogenic alterations typical of myelodysplastic syndrome or acute myelogenous leukemia.

- Member is not affected by end-organ dysfunction, severe active infection, or any other severe disease that, in the opinion of the provider, would make the member an inappropriate candidate for treatment with the requested medication.
- Member will be assessed and monitored for evidence of engraftment failure, cytopenia, infection, hepatic veno-occlusive disease, and malignancies as outlined in the manufacturer's prescribing information.
- Member has not received Waskyra or any other gene therapy previously.

# Appendix

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

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

# 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