Draft New Policy: Do Not Implement

Etuvetidigene autotemcel (Waskyra®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

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
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 SPECIALITIES

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)

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

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

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

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

EFFECTIVE DATE

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