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Section:	Prescription Drugs	**Effective Date:**	June 19, 2026
Subsection:	Miscellaneous Products	**Original Policy Date:**	June 19, 2026
Subject:	Waskyra	**Page:**	1 of 3

Last Review Date: December 11, 2026

Waskyra

Description

Waskyra (etuvetidigene autotemcel)

Background
Waskyra is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of patients with Wiskott-Aldrich Syndrome (WAS). Waskyra adds full-length copies of the human WAS complementary deoxyribonucleic acid (cDNA) into patients' hematopoietic stem cells (HSCs) through transduction with WAS lentiviral vector (LVV). After infusion, the genetically modified cells engraft in the bone marrow, repopulate the hematopoietic compartment, and produce biologically active lymphoid and myeloid progenitors whose progeny express WAS protein (WASP). WASP regulates the structural protein actin in blood cells (1).

Regulatory Status
FDA approved indication: Waskyra is an autologous hematopoietic stem cell-based gene therapy 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 (1).

Waskyra carries warning for hypersensitivity and infusion-related reactions, engraftment failure, cytopenias, serious infections, transmission of an infectious agent, hepatic veno-occlusive disease, risk of oncogenesis, interference with HIV testing, and blood, organ, tissue and cell donation. Monitor patient for signs and symptoms of these possible reactions and administer appropriate treatment accordingly. Do not screen patients who have received Waskyra for HIV infection using a PCR-based assay, as it may result in a false positive. Advise patients not to donate blood, organ, tissue or cells for transplantation at any time in the future (1).

The safety and effectiveness of Waskyra in pediatric patients less than 6 months of age has not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Waskyra may be considered **medically necessary** if the conditions indicated below are met.

Waskyra may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 6 months of age or older

Diagnosis

Patient must have the following:

Wiskott-Aldrich Syndrome (WAS)

AND ALL of the following:

a. Patient has a mutation in the WAS gene
b. Hematopoietic stem cell transplantation (HSCT) is appropriate
c. **NO** previous hematopoietic stem cell gene therapy
d. **NO** suitable human leukocyte antigen (HLA)-matched related stem cell donor available

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity One infusion (only one PA approval for one infusion per lifetime)

Rationale

Summary

Waskyra is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of patients with Wiskott-Aldrich Syndrome (WAS). Waskyra carries warning for hypersensitivity and infusion-related reactions, engraftment failure, cytopenias, serious infections, transmission of an infectious agent, hepatic veno-occlusive disease, risk of oncogenesis, interference with HIV testing, and blood, organ, tissue and cell donation. The safety and effectiveness of Waskyra in pediatric patients less than 6 months of age has not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Waskyra while maintaining optimal therapeutic outcomes.

References

1. Waskyra [package insert]. Rome, Italy: Fondazione Telethon ETS; December 2025.

Policy History

Date	Action
June 2026	Addition to PA

Keywords

This policy was effective with interim approval on June 19, 2026 and will be reviewed by the FEP® Pharmacy and Medical Policy Committee on December 11, 2026.