DR.027.A Waskyra (etuvetidigene autotemcel)

Original Implementation Date: 12/07/2026
Version [A] Date: 12/07/2026
PARP Approval Date: 09/21/2026
Last Reviewed Date: 10/21/2026

Thank you for being a valued provider for members in one or more of our health plans: Jefferson Health Plans Medicare Advantage, Jefferson Health Plans Individual and Family Plans, Jefferson Health Plans CHIP, and/or Jefferson Health Plans EverWell (our Medicaid plan).

***** NOTIFICATION OF PENDING POLICY IMPLEMENTATION *****

Please note that this Policy Bulletin will be implemented on 12/07/2026. This document provides a 30-day notification of its pending implementation and is not currently implemented.

PRODUCT VARIATIONS

This policy applies to all Jefferson Health Plans lines of business unless noted below.

Gene therapy is a benefit exclusion for Individual and Family (ACA) product lines and therefore, non-covered.

POLICY STATEMENT

The plan considers Waskyra (etuvetidigene autotemcel) medically necessary for its FDA approved indications when the prior authorization criteria listed in this policy are met.

FDA APPROVED INDICATIONS

Gene Therapy is the introduction, removal, or change in the content of a person's genetic code with the goal of treating or curing a disease. It includes therapies such as gene transfer, gene modified cell therapy, and gene editing.

WASKYRA™ is an autologous hematopoietic stem cell–based gene therapy indicated for the treatment of pediatric patients aged ≥6 months and adults with Wiskott-Aldrich Syndrome (WAS) who have a confirmed mutation in the *WAS* gene. Use is appropriate in patients for whom hematopoietic stem cell transplantation (HSCT) is clinically indicated and who lack a suitable human leukocyte antigen (HLA)-matched related donor.

OFF-LABEL USE

N/A

PRIOR AUTHORIZATION CRITERIA

Waskyra™ (etuvetidigene autotemcel) may be considered medically necessary when **ALL** of the following criteria are met:

1. Member is 6 months of age or older **AND**

2. Diagnosis of severe Wiskott-Aldrich syndrome is confirmed by a pathogenic WAS gene variant genetic testing and at least one of the following criteria:

    a. Severe WAS mutation ("nonsense mutations, deletions and insertions resulting in absent WAS protein [WASP] expression or truncated WASP.")
    b. Absence of Wiskott Aldrich syndrome protein expression (Absent defined as WASP expression < 5%)
    c. Severe clinical score (Zhu clinical score ≥ 3;

    AND

3. 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" **AND**

4. Member has not received Waskyra or any other gene therapy previously **AND**

5. Treatment is prescribed by specialists experienced in managing Wiskott-Aldrich syndrome **AND**

6. Member has negative HIV and Hepatitis C RNA testing **AND**

7. Member has no known cytogenetic alterations typical of myelodysplastic syndrome/acute myeloid leukemia **AND**

8. Member does not have neoplasia (apart from local skin cancer) **AND**

9. 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; **AND**

10. 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.

RENEWAL CRITERIA

Waskyra™ is administered as a single infusion and is intended for one-time use only.

DOSAGE AND ADMINISTRATION

Per package labeling.

RISK FACTORS/SIDE EFFECTS

Per package labeling.

MONITORING

Per package labeling.

CONTRAINDICATIONS

- Hypersensitivity: Known hypersensitivity or allergic reaction to Waskyra™ or any of its components

- Recent stem cell transplant: Hematopoietic stem cell transplant (HSCT) within the past 6 months or evidence of residual donor cells after transplant.
- Prior gene therapy: Previous treatment with hematopoietic stem cell gene therapy
- Inability to undergo required procedures: Conditions that prevent safe completion of treatment procedures, including:
    - Conditioning (chemotherapy prior to infusion).
    - Stem cell mobilization.

BLACK BOX WARNING

N/A

CLINICAL EVIDENCE

FDA approval for Waskyra™ is based on data from two open-label, single-arm, multinational clinical studies, along with an expanded access program that included 27 patients with severe Wiskott–Aldrich syndrome (WAS). The results demonstrated substantial and sustained clinical benefit, with significant reductions in key disease manifestations. The rate of severe infections decreased by 93% during the 6- to 18-month post-treatment period compared with the 12 months prior to treatment. Additionally, moderate-to-severe bleeding events were reduced by 60% in the first 12 months following treatment. At four years post-treatment, most patients experienced no moderate or severe bleeding episodes, indicating durable long-term efficacy.

BACKGROUND

Wiskott–Aldrich syndrome (WAS) is a rare genetic disorder of the immune system that primarily affects boys. It is characterized by abnormal immune function and a reduced ability to form blood clots, caused by mutations in the *WAS* gene.

Wiskott–Aldrich syndrome is typically defined by three major features:

- A low number of platelets and reduced platelet size, leading to an increased tendency to bleed
- Recurrent bacterial, viral, and fungal infections
- Eczema, an inflammatory skin condition

The key laboratory findings in Wiskott–Aldrich syndrome involve abnormalities in platelets. In addition to being decreased in number, platelets are unusually small and functionally impaired. Assessment of platelet size and morphology is often one of the quickest and most useful initial tests when Wiskott–Aldrich syndrome is suspected.

The Zhu Clinical Scoring System was developed to quantify the severity of disease in Wiskott-Aldrich syndrome (WAS) and related X-linked thrombocytopenia (XLT) phenotypes. This system assigns scores based on the presence and intensity of key clinical manifestations, including thrombocytopenia, eczema, immunodeficiency, autoimmune disorders, and malignancy, allowing for the stratification of patients along a continuum from mild intermittent XLT (score <1) to classic severe WAS (score 5). A score of 5 indicates the most severe phenotype, traditionally associated with the onset of autoimmune disease or malignancy; however, later adaptations have broadened this definition to include severe refractory thrombocytopenia (with a platelet count of $\leq 10 \times 10^9$/L). In clinical practice, the Zhu system aids in categorizing WAS-related disorders by phenotype, guiding prognosis, determining the timing and urgency of interventions such as hematopoietic stem cell transplantation (HSCT) or gene therapy, and monitoring disease progression, particularly during the first two years of life when the phenotype may change.

Table: 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	

Feature					Score	Phenotype
Thrombocytopenia	**Eczema**	**Immunodeficiency**	**Autoimmune Disorders**	**Malignancy**		
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 with autoimmunity and/or malignancy

- XLN = X-linked neutropenia; XLT = X-linked thrombocytopenia
- Source : Bosticardo et al., 2009 ; Chandra et al., 2004

Treatment for Wiskott–Aldrich syndrome focuses on managing symptoms and, when possible, curing the disease by correcting the underlying genetic defect.

Waskyra™ (etuvetidigene autotemcel) is a gene therapy used to treat patients with Wiskott–Aldrich syndrome (WAS). It is a one-time, personalized treatment designed to correct the underlying genetic defect.

CODING

Note: *The Current Procedural Terminology (CPT®), Healthcare Common Procedure Coding System (HCPCS), and the 10th revision of the International Statistical Classification of Diseases and Related Health Problems (ICD-10) codes that may be listed in this policy are for reference purposes only. Listing of a code in this policy does not imply that the service is covered and is not a guarantee of payment. Other policies and coverage guidelines may apply. When reporting services, providers/facilities should code to the highest level of specificity using*

the code that was in effect on the date the service was rendered. This list may not be all inclusive.

CPT® is a registered trademark of the American Medical Association.

CPT Code	Description
N/A	N/A

HCPCS Code	Description
J3386	Injection, etuvetidigene autotemcel, per treatment

ICD-10 Codes	Description
N/A	N/A

DISCLAIMER

Approval or denial of payment does not constitute medical advice and is neither intended to guide nor influence medical decision making. Policy Bulletins are developed to assist in administering plan benefits and constitute neither offers of coverage nor medical advice. This Policy Bulletin may be updated and therefore is subject to change.

For Jefferson Health Plans Everwell and Jefferson Health Plans Chip products. Any requests for services that do not meet criteria set in PARP will be evaluated on a case-by-case basis.

POLICY HISTORY

This section provides a high-level summary of changes to the policy since the previous version.

Summary	Version	Version Date
New policy.	A	TBD

REFERENCES

1. Waskyra package insert: https://www.fda.gov/media/190096/download?attachment

2. Wiskott-Aldrich Foundation: https://www.wiskott.org/About-WAS/treatment-of-was/waskyra

3. About WAS (Wiskott-Aldrich Syndrome): https://www.wiskott.org/About-WAS

4. Cleveland Clinic-Wiskott-Aldrich Syndrome: https://my.clevelandclinic.org/health/diseases/wiskott-aldrich-syndrome

5. Drugs.com Waskyra: https://www.drugs.com/waskyra.html

6. Waskyra (etuvetidigene autotemcel) last updated: **Aug 07, 2026** https://www.waskyra.com/