Pharmacy Drug Policy

SUBJECT: Waskyra (etuvetidigene autotemcel) – Medical Benefit
POLICY NUMBER: PHARMACY-148
EFFECTIVE DATE: 11/2026
LAST REVIEW DATE: 11/24/2026

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Wiskott-Aldrich Syndrome (WAS) is an X-linked genetic disorder characterized by immunodeficiency, thrombocytopenia with small platelets, eczema and increased risk of autoimmunity and malignancies. Variation in the disease is dependent on the gene mutations and can range from a severe phenotype (classic WAS) to milder phenotypes (X-linked thrombocytopenia and X-linked neutropenia). Mutations in the WAS gene may lead to deficiency in Wiskott-Aldrich Syndrome Protein (WASP), an essential protein for the functioning of specific blood cells. Immune T and B cells that lack WASP do not have a proper immunological synapsed formation, cell migration, and cytotoxicity. As a result, WAS leads to a weaker immune response and affects the formation of functional platelets. Disease incidence is roughly four in one million live male births in the U.S. According to the Wiskott-Aldrich Foundation, there are approximately 500 patients diagnosed with WAS in the U.S.

Diagnosis of WAS can be confirmed through genetic testing. Classification of disease is based on the Zhu clinical scoring system (Table 1). Patients with XLT (X-linked thrombocytopenia) or X-linked neutropenia (XLN) are considered to have milder disease.
Allogeneic hematopoietic stem cell transplantation (HSCT) is the standard of care for WAS. It is a potential curative treatment if an adequate donor is available. Other treatment options are supportive measures and include administration of immunoglobulins, platelet transfusions to increase platelet counts, antibiotic and antiviral therapies to prevent infection, topical products to manage eczema and medications (e.g. rituximab or IVIG) to manage autoimmunity. Splenectomy is used in some special cases of uncontrolled bleeding but should be avoided otherwise, due to risk of serious infections.

Waskyra (etuvetidigene autotemcel) is an autologous hematopoietic stem cell-based gene therapy approved for the treatment of patients aged 6 months and older with WAS who have a mutation in the WAS gene for whom HSCT is appropriate and no suitable human leukocyte antigen-matched related hematopoietic stem cell donor is available. This gene therapy modifies patients' CD34+ hematopoietic stem and progenitor cells by lentiviral vector-mediated integration of the functional WAS complementary deoxyribonucleic acid (cDNA) sequence. Following infusion, the genetically modified cells impart themselves in the bone marrow to repopulate the hematopoietic compartment and produce biologically active lymphoid and myeloid progenitors which can produce cells that express WASP. It is a single-dose intravenous infusion with a recommended dose of 7×10^6 CD34+ cells per kg.

POLICY:

Waskyra (etuvetidigene autotemcel) - Medical
1. Must be at least 6 months of age and not greater than 65 years of age **AND** 2. Prescribed by or in consultation with an immunologist, hematologist, or presciber that specializes in the treatment of Wiskott Aldrich Syndrome at a qualified treatment center **AND** 3. Must have documentation of a diagnosis of Wiskott Aldrich Syndrome confirmed by the presence of a WAS gene mutation **AND** documentation of one of the following criteria: a. Severe Wiskott-Aldrich Syndrome (WAS) gene mutation, defined by literature data (genotype/phenotype studies) (see Appendix for details) **OR** b. Absence of WASP expression, assessed by flow cytometry or western blot **OR** c. Severe clinical score (Zhu clinical score of ≥3) (see Appendix for details) **AND** 4. Patient has been screened and found negative for active infections of the following: hepatitis B virus (HBV), hepatitis C virus (HCV), human immunodeficiency virus 1&2 (HIV-1/HIV-2), and tuberculosis prior to collection of cells (documentation of laboratory results taken within the past 3 months is required) **AND** 5. Patient will not receive therapy concomitantly with anti-retroviral therapy for at least one month prior to mobilization until at least 7 days after infusion of Waskyra **AND** 6. The patient must be eligible to undergo hematopoietic stem cell transplant (HSCT) **AND** 7. Must have documentation that the patient does not have a human leukocyte antigen (HLA)-identical related donor available for hematopoietic stem cell transplant **AND** 8. Patients must not have myelodysplasia, cytogenetic alterations typical of myelodysplastic syndromes and acute myeloid leukemia, or other serious hematological disorders, based on standard clinical evaluation (documentation within the past 3 months is required). Typical baseline evaluation includes but is not limited to complete blood count with differential, peripheral smear, clinical assessment for cytopenias or marrow dysfunction 9. Waskyra is indicated for one-time single-dose intravenous use only and therefore will not be authorized for retreatment. Retreatment will be considered experimental/investigational when any FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered. 10. Waskyra will not be authorized for any of the following: a. Malignant neoplasia (except local skin cancer) or a documented history of hereditary cancer syndrome b. End-organ dysfunction c. Undergone allogeneic hematpoietic stem cell transplantation in the previous 6 months d. Patients who underwent an allogeneic hematopoietic stem cell transplantation with evidence of residual cells of donor origin 11. Use of Waskyra (etuvetidigene autotemcel) is considered experimental/investigational when the above criteria are not met 12. Use of Waskyra (etuvetidigene autotemcel) to treat other indications, including X-linked thrombocytopenia, will be considered experimental/investigational 13. Approval will be for 6 months to allow one-time administration

APPENDIX:

Zhu Clinical Score

	XLT			WAS			
Clinical scores	0.5	1	2	3	4	5A	5M
Thrombocytopenia	+/-	+	+	+	+	+	+
Eczema	-	-	+/-	+	++	++/-	++/-
Immunodeficiency	-	-	+/-	+	++	++/-	++/-
Autoimmunity	-	-	-	-	-	+	-
Malignancy	-	-	-	-	-	-	+

The score is based on Zhu and colleagues (Zhu et al. 1997), with subsequent refinements (Bosticardo et al., 2009).

Genotype/Phenotype Studies:

https://ashpublications.org/blood/article/104/13/4010/19014/Mutations-of-the-Wiskott-Aldrich-Syndrome-Protein

POLICY GUIDELINES:

1. Utilization Management are contract dependent. Refer to specific contract/benefit language for exclusions.
   a. Coverage criteria may be dependent on the contract renewal date.
   b. Coverage of drugs listed in this policy are contract dependent.
   c. Not all contracts/benefits allow coverage of healthcare professional administered drugs as part of their pharmacy benefit
   d. Not all contracts/benefits cover all medical infusible drugs.
   e. Not all policy criteria may apply if treatment of specific disease states or conditions is not a covered benefit.
2. Clinical documentation must be submitted for each request (initial and recertification [if applicable]) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments and treatment history, diagnostic testing, laboratory test results, genetic testing or biomarker results, imaging, and other objective or subjective measures of clinical benefit.
3. This policy is based on available evidence as of the last review date. Coverage determinations are subject to applicable plan documents, state and federal regulations, and individual patient circumstances. This policy does not constitute medical advice.
4. For commercial contracts, medical necessity determinations align with the Certificate of Coverage issued by the Health Plan, which states that covered services must be clinically appropriate and not primarily for the convenience of the member, the member's family, or the provider.
5. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
6. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. In the absence of a Medicare National Coverage Determination (NCD), Local Coverage Determination (LCD), or other Medicare coverage guidance, CMS permits a Medicare Advantage Organization (MAO) to establish its own coverage determinations in accordance with 42 CFR § 422.101(b)(6). Indications that have not

been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan. Step therapy requirements may be imposed in addition to LCD/NCD requirements.

7. Unless otherwise indicated within drug specific criteria, the drugs listed in this policy are administered by a healthcare professional and therefore are covered under the medical benefit.
8. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
9. This policy is subject to ongoing revision. Newly marketed drugs and existing drugs with new indications may be subject to prior authorization until formal coverage criteria are established. Inclusion of a drug in this policy does not guarantee its current availability on the market, as some agents may be discontinued, withdrawn, or otherwise unavailable. As product status changes, drugs may be removed from the policy.
10. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html
11. Approval timeframes may be extended to align authorization periods for drugs administered as part of a multidrug regimen, including when regimen components are administered across different benefits. This provision does not apply when the approval timeframe reflects a drug-specific limitation on duration of therapy
12. The following applies to all gene and cellular therapies unless otherwise specified within the drug-specific coverage criteria:
    a. Administration, Retreatment, and Treatment with Additional or Other Gene/Cellular Therapies
        i. One-Time Administration
            1. Most gene and cellular therapies, whether autologous, allogeneic (“off-the-shelf”), or in vivo gene-transfer therapies, are designed and studied as one-time treatments.
            2. Repeat dosing, reinfusion, or sequential therapy with other gene or cellular products has not been established as safe, effective, or clinically appropriate.
        ii. Retreatment/Repeat Administration
            1. Retreatment with the same gene or cellular therapy product is considered experimental and investigational because:
                a. Clinical trials evaluated these therapies as single-administration interventions
                b. Safety, efficacy, and durability of a second administration have not been established
                c. Risks of immune activation, insertional mutagenesis, or vector immunity may be increased with repeat dosing
        iii. Treatment with an Additional or Other Gene/Cellular Therapy
            1. Treatment with an additional or different gene or cellular therapy after prior exposure to any gene or cellular therapy is also considered experimental and investigational, unless supported by evidence demonstrating (a-c):
                a. Anticipated clinical benefit beyond available standard therapies
                b. Safety of sequential administration
                c. Justification for selecting a second gene/cellular intervention after a prior one
            2. This includes, but is not limited to:
                a. Switching between CAR-T products (e.g., CD19 → CD19 or CD19 → BCMA)

b. Switching between autologous and allogeneic cellular therapies
c. Sequential use of CAR-T, TCR-T, NK-cell therapies, or other genetically engineered cell therapies
d. Receiving a gene therapy after previous gene or cellular therapy exposure
e. Receiving an in vivo gene therapy following any prior vector-based therapy

iv. Prior Gene/Cell Therapy Exposure
1. An individual is generally not eligible for additional gene or cellular therapy if they have previously received:
a. Any autologous cellular therapy (e.g., CAR-T, TCR-T, TIL),
b. Any allogeneic genetically modified cellular therapy,
c. Any in vivo gene therapy (e.g., AAV, lentiviral vector)
d. Any ex vivo gene-modified cell product
e. Are being considered for any other gene or cellular therapy without documented evidence supporting safety and anticipated benefit.

13. The requested site of care may impact approval timeframe and is subject to review.

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.
CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:
Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).
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HCPCS: J3386 Waskyra

UPDATES:

Date	Revision
11/24/2026	Created
11/12/2026	P&T Committee Approval of Criteria

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