Medical Policy

BlueCross BlueShield Kansas
An Independent licensee of the
Blue Cross Blue Shield Association

Title: Waskyra

Professional / Institutional
Original Effective Date: September 10, 2026
Latest Review Date:
Current Effective Date: September 10, 2026

State and Federal mandates and health plan member contract language, including specific provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage. To verify a member's benefits, contact Blue Cross and Blue Shield of Kansas Customer Service.

The BCBSKS Medical Policies contained herein are for informational purposes and apply only to members who have health insurance through BCBSKS or who are covered by a self-insured group plan administered by BCBSKS. Medical Policy for FEP members is subject to FEP medical policy which may differ from BCBSKS Medical Policy.

The medical policies do not constitute medical advice or medical care. Treating health care providers are independent contractors and are neither employees nor agents of Blue Cross and Blue Shield of Kansas and are solely responsible for diagnosis, treatment and medical advice.

If your patient is covered under a different Blue Cross and Blue Shield plan, please refer to the Medical Policies of that plan.

POLICY AGENT SUMMARY – MEDICAL PRIOR AUTHORIZATION

Indication	Dose
Wiskott-Aldrich Syndrome (WAS)	The minimum recommended dose of Waskyra is 7×10^6 CD34+ cells/kg based on the member's body weight at the time of infusion. The maximum volume of Waskyra to be administered should remain < 20% of the member's estimated plasma volume. Mobilization and apheresis • Mobilize hematopoietic stem and progenitor cells (HSPC) using G-CSF with plerixafor according to established protocols. • Perform apheresis to collect CD34+ cells required for Waskyra manufacturing following the mobilization procedure. • Collect and cryopreserve a back-up supply of CD34+ stem cells containing at least 3 x 10^6 CD34+ cells/kg from the member. Complete this collection before initiating reduced intensity conditioning and Waskyra infusion. • Store the back-up collection for potential rescue treatment in the following scenarios: Waskyra compromise after reduced intensity conditioning initiation but before scheduled infusion, primary engraftment failure, or prolonged bone marrow aplasia following Waskyra treatment. Pre-treatment and conditioning • Administer rituximab (anti-CD20 monoclonal antibody) approximately 22 days before Waskyra administration to deplete autoreactive B-cells and provide pre-emptive treatment for potential lymphoproliferative disorder due to Epstein Barr Virus infection which is a risk factor in WAS members. • Administer busulfan and fludarabine for reduced intensity conditioning before infusion of Waskyra to promote engraftment of the genetically modified autologous CD34+ cells. • Do not begin conditioning until the complete set of infusion bag(s) constituting the dose of Waskyra has been received and stored at the administration site. Confirm availability of the back-up collection. Premedication • Administer intravenous chlorpheniramine (0.2 mg/kg, max. dose 10 mg), or an equivalent 15-30 minutes before the infusion of Waskyra to reduce the possibility of an allergic reaction to the infusion.

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for ONE dose total.
- Renewal: Prior authorization validity may NOT be renewed.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 1 billable unit (1 treatment of up to 8.05 $x10^8$ $CD34^+$ cells/mL)

III. Initial Approval Criteria

Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax**.** Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines.

Prior authorization validity is provided in the following conditions:

Wiskott-Aldrich Syndrome (WAS) † Φ [1-5]

- Member is at least 6 months of age; **AND**
- Member has a diagnosis of Wiskott-Aldrich Syndrome (WAS) as evidenced by a mutation in the WAS gene, as confirmed by gene mutation testing, and has one of the following criteria:
  - Severe phenotype WAS mutation; **OR**
  - Signs and symptoms consistent with classic, severe WAS phenotype *(i.e., Zhu-Clinical- or WAS-Severity- Score of three or greater)*; **OR**
  - Absent WAS protein (WASP) expression; **AND**
- Member is a candidate for allogenic hematopoietic stem cell transplant (HSCT) and has not had prior allogenic HSCT; **AND**
- Member does not have a suitable human leukocyte antigen (HLA)-matched related stem cell donor; **AND**
- Member does NOT have any FDA labeled contraindications to the requested agent (e.g., hypersensitivity to the active substance or to any of the excipients, previous treatment with HSCT within 6 months prior to screening or HSCT with evidence of residual donor cells, previous treatment with hematopoietic stem cell gene therapy, contraindications to the mobilization and the conditioning regimen, etc.); **AND**
- Used as single agent therapy (not applicable to lymphodepleting/conditioning or bridging therapy while awaiting manufacture); **AND**

- Member will be monitored for signs and symptoms of infection before and after infusion, with prophylactic antimicrobials administered according to local guidelines; **AND**
- Member will be monitored to maintain immunoglobulin G serum level above 5 g/L**; AND**
- Member will be monitored for signs and symptoms of veno-occlusive disease including assessment of liver function tests during the first month following infusion; **AND**
- Member will undergo mobilization with granulocyte-colony-stimulating factor (G-CSF) and plerixafor prior to apheresis; **AND**
- Member will be monitored for lentiviral vector (LVV)-mediated insertional oncogenesis and secondary malignancies following treatment as clinically indicated; **AND**
- Member has been screened and found negative for human immunodeficiency virus (HIV) in accordance with clinical guidelines prior to mobilization and apheresis *(**Note:** Members who have received Waskyra may test positive by polymerase chain reaction (PCR) assays for HIV due to LVV provirus insertion, resulting in a false-positive PCR assay test result for HIV. Therefore, members who have received Waskyra should not be screened for HIV infection using a PCR-based assay.)*; **AND**
- Member will not be administered live-virus vaccinations until immune reconstitution is completed; **AND**
- Member will not receive anti-retroviral medications *(**Note:** Members receiving anti-retroviral medications should stop therapy for at least one month prior to mobilization and until at least 7 days after Waskyra infusion).*

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria [1]

- Duration of authorization has not been exceeded (refer to Section I)

Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

CLINICAL RATIONALE

See package insert for FDA preshttps://dailymed.nlm.nih.gov/dailymed/index.cfm

CODING

The following codes for treatment and procedures applicable to this policy are included below for informational purposes. This may not be a comprehensive list of procedure codes applicable to this policy.

Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

The code(s) listed below are medically necessary ONLY if the procedure is performed according to the "Policy" section of this document.

HCPCS Code(s):

- J3386 – Injection, etuvetidigene autotemcel, per treatment; 1 billable unit = 1 treatment

NDC:

- Waskyra is supplied in 1 to 8 infusion bags containing a frozen suspension of genetically modified autologous cells enriched for CD34+ cells. Each bag contains 10 to 20 mL of suspension, 50mL infusion bag, overwrap, and metal cassette: NDC Not Available

REVISIONS	
Posted: 08-11-2026 Effective: 09-10-2026	New medical policy added to the bcbsks.com web site. Policy is maintained by Prime Therapeutics LLC.

REFERENCES

1. Waskyra [package insert]. Rome, Italy; Fonadazione Telethon, ETS; December 2025. Accessed June 2026.
2. Bonilla FA, Khan DA, Ballas ZK, et al. Practice Parameter for the diagnosis and management of primary immunodeficiency. J Allergy Clin Immunol 2015 Nov;136(5):1186-205.e1-78.
3. ClinicalTrials.gov. A Phase I/II Clinical Trial of Hematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome https://clinicaltrials.gov/study/NCT01515462.
4. 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 May;6(5):e239-e253. doi: 10.1016/S2352-3026(19)30021-3. Epub 2019 Apr 10. PMID: 30981783; PMCID: PMC6494976.
5. Chandra S, Nagaraj CB, Sun M, et al. WAS-Related Disorders. GeneReviews. https://www.ncbi.nlm.nih.gov/books/NBK1178/ (Accessed on June 5, 2026).