



Nonprofit corporations and independent licensees
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

P&T Date: 02/12/2026

Waskyra™ (etuvetidigene autotemcel)

HCPCS: J3590

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

- A. Coverage of the requested drug is provided when all the following are met:
 - a. FDA approved indication
 - b. FDA approved age
 - c. Diagnosis of Wiskott-Aldrich syndrome (WAS) confirmed via genetic testing showing a mutation of the WAS gene with one of the following:
 - i. Absence of WAS protein expression
 - ii. WAS clinical score greater than or equal to 3
 - d. Must not have had a prior hematopoietic stem cell transplant (HSCT) or currently be eligible for a HSCT with an HLA matched family donor. For children younger than 5 years of age, no suitable 10/10 matched unrelated donor or 6/6 unrelated cord blood donor
 - e. Must not have presence of HIV-1, HIV-2, hepatitis B, or hepatitis C infection
 - f. Have not received prior treatment with any gene therapy or are being considered for treatment with any other gene therapy for WAS
 - g. The requesting physician attests to providing clinical outcome information within the appropriate provider portal as requested by BCBSM
 - h. Trial and failure, contraindication, OR intolerance to the preferred drugs as listed in BCBSM/BCN's utilization management medical drug list
- B. Quantity Limitations, Authorization Period and Renewal Criteria
 - a. Quantity Limits: Align with FDA recommended dosing
 - b. Authorization Period: 3 months with the allowance of only one dose per lifetime
 - c. Renewal Criteria: Not applicable as no further authorization will be provided

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

This policy and any information contained herein is the property of Blue Cross Blue Shield of Michigan and its subsidiaries, is strictly confidential, and its use is intended for the P&T committee, its members and BCBSM employees for the purpose of coverage determinations.

Background Information:

- Wiskott-Aldrich syndrome (WAS) is a rare X-linked disorder caused by a mutation on the WAS gene. It is characterized by a dysfunctional WAS protein resulting in thrombocytopenia. The disease occurs almost exclusively in males.
- The symptoms of WAS typically begin in infancy and include bloody diarrhea, abnormal bleeding episodes, petechia, eczema, and recurrent ear infections. Thrombocytopenia, in association with small platelet size, is usually present at birth, but platelet counts may initially be near normal. Intracranial bleeding is a possible life-threatening complication. Children with WAS have an increased risk for recurrent bacterial and viral infections. Autoimmune disorders are also common in those who survive and include hemolytic anemia, arthritis, vasculitis, and kidney and liver damage. The risk for developing an autoimmune disorder increases with age. In addition, there is an increased risk of developing lymphomas, especially after exposure to Epstein-Barr virus.
- The 2015 practice parameter for the diagnosis and management of primary immunodeficiency state WAS should be suspected in all male patients with clinically significant thrombocytopenia and small platelets with platelet volumes ranging from 3.8 fL to 5.0 fL. For a definitive molecular diagnosis, finding a known deleterious WAS mutation and/or abnormal WAS protein expression is necessary.
- A disease scoring system has been created that allows for clinical categorization of patients with WAS and may be useful in predicting disease severity and outcome of hematopoietic stem cell transplantation (HSCT). A score of 1 or 2 defines patients with X-linked thrombocytopenia (XLT), a score of 3 to 4 identifies patients with classic WAS, and a score of 5 is reserved for patients with either XLT or WAS after developing autoimmunity and/or malignancies. The clinical phenotype of the disease evolves over time and is often incomplete in males younger than two years of age. Because of this, WAS scores should not be used during infancy to predict disease severity. An established WAS score describes current disease severity and does not reflect the possibility of disease progression to a more severe form.
- Guidelines recommend HSCT be seriously considered for patients less than 5 years of age with suitable stem cell donors. If no stem cell donor is available, patients should be treated with supportive care including prophylaxis against PCP and appropriate antimicrobial therapy for infections, intravenous immunoglobulin, routine childhood immunizations, and treating eczema with topical steroid therapy. Treatment of significant bleeding sometimes includes platelet transfusions.
- Waskyra is the first disease modifying therapy approved for the treatment of pediatric patients aged 6 months and older and adults with WAS who have a mutation in the WAS gene for whom HSCT is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.
- Safety and efficacy of Waskyra were evaluated in two clinical studies, study 1 and study 2, as well as an expanded access program. Study 1 was a prospective, open-label, single-arm trial of 8 patients with WAS that compared efficacy of Waskyra fresh formulation compared to 12 month pre-treatment outcomes. Study 2 is an ongoing, open-label, single-arm trial of 10 patients with WAS that is evaluating the efficacy of Waskyra cryopreserved formulation compared to 12 month pre-treatment outcomes. Subjects must have had a genetic mutation confirming diagnosis of WAS with at least one of the following: absence of WAS protein expression; a WAS clinical score greater than or equal to 3; or a severe WAS mutation. Participants did not have a matched HLA-identical sibling donor. Following treatment with rituximab and a reduced-intensity conditioning regimen of busulfan and fludarabine, patients received one intravenous infusion of Waskyra. The primary endpoints were the rate of severe infections during the 6 to 18 month period after Waskyra infusion compared with the 12-month pre-treatment period, and the rate of moderate or severe bleeding episodes during the 12-month period after Waskyra infusion compared with the 12-month pre-treatment period. The rate of severe infections decreased from 2.0 (95% CI: 1.50, 2.61) infections per patient year observation (PYO) in the 12 months pre-treatment period to 0.2 (95% CI: 0.04, 0.40) per PYO infections in 6 - 18

months post-gene therapy. The rate of moderate and severe bleeding events decreased from 2.0 (95% CI: 1.50, 2.61) events per PYO in the 12 months pre-treatment to 0.8 (95% CI: 0.49, 1.22) events per PYO in the 12 months after treatment

- Waskyra has not been studied and there is no data to support use in combination with other gene therapies indicated for use in WAS.
- Provider portal platforms allow the capture of clinical outcome information for patients on select high-cost treatments, such as gene and cellular therapies. If a patient meets medical necessity as defined by this policy and is approved for treatment, the requesting physician must attest to providing clinical outcome information within the appropriate provider portal at the requested cadence.

References:

1. Waskyra [prescribing information]. Rome, Italy: Fondazione Telethon ETS; December 2025.
2. Clinicaltrials.gov. A clinical study to evaluate the use of a cryopreserved formulation of OTL-103 in subjects with wiskott-aldrich syndrome (NCT03837483). Available at: <https://clinicaltrials.gov/study/NCT03837483>. Accessed on December 11, 2025.
3. Clinicaltrials.gov. Gene therapy for wiskott-aldrich syndrome (NCT01515462). Available at: <https://clinicaltrials.gov/study/NCT01515462>. Accessed on December 11, 2025.
4. Ferrua F, Cicalese MP, Galimbert 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 Apr 10; 6 (5): e239 – 53.
5. National Organization for Rare Disorders. WAS-related disorders. 2008 Feb 15. Available at: <https://rarediseases.org/rare-diseases/was-related-disorders/>. Accessed on December 11, 2025.
6. Bonilla FA, Khan DA, Ballas ZK, et al. Practice parameter for the diagnosis and management of primary immunodeficiency. *J Allergy Clin Immunol*. 2015 Sept 12; 136 (5): 1186 – 205.
7. Cavanaugh C, Ochs HD, & Buchbinder D. Diagnosis and clinical management of Wiskott–Aldrich syndrome: current and emerging techniques. *Exp Rev Clin Immunol*. 2022 May 19; 18 (6): 609 – 23.
8. Imai K, Morio T, Zhu Y, et al. Clinical course of patients with WASP gene mutations. *Blood*. 2004 Jan 15; 103 (2): 456 – 64.
9. Albert MH, Notarangelo LD, & Ochs HD. Clinical spectrum, pathophysiology and treatment of the Wiskott-Aldrich syndrome. *Curr Opin Hematol*. 2011 Jan; 18 (1): 42 - 8.
10. Ochs HD & Thrasher AJ. The Wiskott-Aldrich syndrome. *J Allergy Clin Immunol*. 2006 April; 117 (4): 725 – 38.

Policy History												
#	Date	Change Description										
1.2	Effective Date: 02/12/2026	New policy - this criteria replaces previously approved preliminary criteria										
1.1	Effective Date: 01/15/2026	UM medical management system update for MAPPO and BCNA <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	Yes	BCNA	Yes
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
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BCNA	Yes											
1.0	Effective Date: 12/22/2025	UM medical management system update for BCBS and BCN <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	No	BCNA	No
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BCN	Yes											
MAPPO	No											
BCNA	No											

* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.

Blue Cross Blue Shield/Blue Care Network of Michigan

Medication Authorization Request Form



This form is to be used by participating physicians to obtain coverage for **drugs covered under the medical benefit**. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

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PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	Name
ID Number	Specialty
D.O.B. ☐ Male ☐ Female	Address
Diagnosis	City /State/Zip
Drug Name	Phone/Fax: P: () - F: () -
Dose and Quantity	NPI
Directions	Contact Person
Date of Service(s)	Contact Person Phone / Ext.

STEP 1: DISEASE STATE INFORMATION

- Is this request for: ☐ Initiation ☐ Continuation Date patient started therapy: _____
- Administered by patient or a medical professional? ☐ patient (self) ☐ health care professional (physician, nurse, etc.)
- Site of administration? ☐ Provider office/Home infusion ☐ Other: _____
☐ Hospital outpatient facility (go to #4) Reason for Hospital Outpatient administration: _____
☐ Hospital inpatient facility for Car-T therapy only (for example: Kymriah, Yescarta, or Tecartus) (go to #5)
- Please specify location of administration if hospital outpatient infusion: _____
- Please specify location of administration if hospital inpatient infusion: _____
- Please provide the NPI number for the place of administration: _____
- Initiation AND Continuation of therapy:**
 - What is the patient's diagnosis? _____
 - What other medication has the patient received for their condition? Please list _____
 - Please describe the response to previous therapies: _____
 - Will the patient be receiving any other treatment for the listed condition while on this medication? Please list: _____
 - Please list any labs values important for diagnosing or monitoring this patient's condition: _____
- Continuation of therapy:**
 - Has the patient progressed while on this medication? ☐ yes ☐ no
 - How has the patient's condition changed while on this medication?

☐ Improved: Please describe: _____
 ☐ Stable: please describe: _____
 ☐ Worsened; Please describe: _____
 ☐ Other; Please describe: _____

Chart notes are required for the processing of all requests. Please add any other supporting medical information necessary for our review (required)

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name	Physician Signature	Date
Step 2: Checklist	☐ Form Completely Filled Out ☐ Provide chart notes	☐ Attach test results
Step 3: Submit	By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979	By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320

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7/26/2018, 9/18/2018; 1/31/2020; 3/17/2020; 8/9/2021