Waskyra™ (etuvetidigene autotemcel)
(Intravenous)

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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)

V. Dosage/Administration [1]

Indication	Dose
Wiskott-Aldrich Syndrome (WAS)	The minimum recommended dose of Waskyra is 7×10^6 CD34+ cells/kg based on the memeber'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.

Indication	Dose
	• 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. <u>Pre-treatment and conditioning</u> • 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. <u>Premedication</u> • 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.
***Note**: When more than one bag of Waskyra is needed, prior to infusion it should be ensured that the volume of product to be infused is compatible with the recommended limit of DMSO, i.e., the total volume of DMSO administered should remain < 1% of the member's estimated plasma volume. The maximum volume of Waskyra to be administered should therefore remain < 20% of the member's estimated plasma volume. Also, when more than one bag of Waskyra is needed, only one bag of medicinal product should be infused at a time.*	

VI. Billing Code/Availability Information

<u>HCPCS Code:</u>

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

<u>NDC:</u>

- 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

VII. 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).

Appendix A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime's assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	No: PA not a priority
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

Appendix 1 – Covered Diagnosis Codes (All Products)

ICD-10	ICD-10 Description
D82.0	Wiskott-Aldrich syndrome

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC