New Hampshire Medicaid Fee-for-Service Program
Waskyra (etuvetidigene autotemcel) Criteria

Approval Date: May 12, 2026

Medications

Brand Names	Generic Names	Indication
Waskyra	etuvetidigene autotemcel	Treatment of pediatric patients 6 months and older and adults with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available

Criteria for Approval

1. Patient is 6 months of age or older; **AND**
2. Patient has a confirmed diagnosis of Wiskott-Aldrich Syndrome (WAS) as confirmed by molecular genetic testing and has severe phenotype WAS mutation; **AND**
3. Patient does NOT have a medically-eligible 10/10 human leukocyte antigen (HLA)-identical matched stem cell donor for transplant (Note: requests when an HLA-identical donor is identified, but mobilized peripheral blood or bone marrow hematopoietic stem cell collection is not feasible will be reviewed on a case-by-case basis); **AND**
4. Patient has NOT received a prior allogeneic hematopoietic stem cell transplant (HSCT) within 6 months prior to screening (or has, but is without evidence of residual donor cells present), and is a candidate for autologous stem cell transplantation (e.g., adequate renal and hepatic function);**AND**
5. Used as single agent therapy (not applicable to lymphodepleting/conditioning or pre-emptive anti-cluster of differentiation [CD]20 therapy while awaiting manufacture); **AND**
6. Patient has NOT received prior hematopoietic stem cell–based gene therapy; **AND**
7. Patient does NOT have a history of hypersensitivity to dimethyl sulfoxide (DMSO), or the active substance of Waskyra or any of the excipients; **AND**
8. Patient is human immunodeficiency virus (HIV) negative as confirmed by a negative HIV test prior to mobilization*; **AND**
9. Patient will be monitored for signs and symptoms of veno-occlusive disease including assessment of liver function tests during the first month after infusion; **AND**
10. Patient will be monitored for signs and symptoms of serious infection and prophylactic antimicrobials will be administered according to standard local or institutional guidelines; **AND**
11. Patient’s immunoglobulin G serum level is above 5 g/L; **AND**
12. Patients will be monitored for malignancies periodically after treatment; **AND**

13. Patient will have mobilization of stem cells using granulocyte-colony stimulating factor (G-CSF) and plerixafor according to established protocols; **AND**
14. Prescriber has reviewed Waskyra Warnings/Precautions and Drug Interactions will monitor patient status as appropriate; **AND**
15. Females of reproductive potential require a negative serum pregnancy test to be confirmed before the start of mobilization and re-confirmed before conditioning procedures and before administration of Waskyra; **AND**
16. Males capable of fathering a child and females of childbearing age should use an effective method of contraception from start of mobilization through a minimum of 6 months after administration of Waskyra.

Criteria for Denial

Above criteria are not met.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	04/21/2026
Commissioner designee	Approved	05/12/2026