Effective Date: 06/20/2025
Current Effective Date: 06/20/2025
Last P&T Approval/Version: 04/30/2025
Next Review Due By: 04/2026
Policy Number: C29396-A

Niktimvo (axatilimab-csfr)

PRODUCTS AFFECTED

Niktimvo (axatilimab-csfr)

COVERAGE POLICY

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Coverage Guideline must be read in its entirety to determine coverage eligibility, if any. This Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide Molina Healthcare complete medical rationale when requesting any exceptions to these guidelines.

Documentation Requirements:
Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive.

DIAGNOSIS:
Chronic graft-versus-host disease (chronic GVHD)

REQUIRED MEDICAL INFORMATION:
This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. If a drug within this policy receives an updated FDA label within the last 180 days, medical necessity for the member will be reviewed using the updated FDA label information along with state and federal requirements, benefit being administered and formulary preferencing. Coverage will be determined on a case-by-case basis until the criteria can be updated through Molina Healthcare, Inc. clinical governance. Additional information may be required on a case-by-case basis to allow for adequate review. When the requested drug product for coverage is dosed by weight, body surface area or other member specific measurement, this data element is required as part of the medical necessity review. The Pharmacy and Therapeutics Committee has determined that the drug benefit shall be a mandatory generic and that generic drugs will be dispensed whenever available.

A. CHRONIC GRAFT VERSUS HOST DISEASE (cGVHD):
1. Documented diagnosis of symptomatic chronic graft-versus-host-disease
   AND

2. Documentation member has previously received at least 2 prior lines of systemic therapy (i.e., corticosteroids, mycophenolate, cyclosporine, tacrolimus, ruxolitinib, ibrutinib, belumosudil) AND
3. Documentation member weighs is at least 40kg AND
4. Documentation of baseline signs and symptoms (e.g., dry eyes, shortness of breath, rash, mouth sores, tingling sensation, muscle and joint pain, etc.) [DOCUMENTATION REQUIRED]

CONTINUATION OF THERAPY:

A. CHRONIC GRAFT VERSUS HOST DISEASE (cGVHD):

1. Prescriber attests to or clinical reviewer has found no evidence of intolerable adverse effects or drug toxicity (See Appendix) AND
2. Documentation of positive clinical response as demonstrated by low disease activity and/or improvements in the condition's signs and symptoms (e.g., dry eyes, shortness of breath, rash, mouth sores tingling sensation, muscle and joint pain, etc.) [DOCUMENTATION REQUIRED]

DURATION OF APPROVAL:

Initial authorization: 12 months, Continuation of Therapy: 12 months

PRESCRIBER REQUIREMENTS:

Prescribed by or in consultation with a board-certified hematologist, oncologist, or transplant specialist [If prescribed in consultation, consultation notes must be submitted with initial request and reauthorization requests]

AGE RESTRICTIONS:

6 years of age and older

QUANTITY:

0.3 mg/kg every 2 weeks

Maximum Quantity Limits: 35 mg per dose

PLACE OF ADMINISTRATION:

The recommendation is that infused medications in this policy will be for pharmacy or medical benefit coverage administered in a place of service that is a non-inpatient hospital facility-based location.

DRUG INFORMATION

ROUTE OF ADMINISTRATION:

Intravenous

DRUG CLASS:

Colony Stimulating Factor-1 Receptor (CSF-1R) Antibodies

FDA-APPROVED USES:

Indicated for the treatment of chronic graft-versus-host-disease (cGVHD) after failure of at least 2 prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg

COMPENDIAL APPROVED OFF-LABELED USES:

None

APPENDIX

APPENDIX:

Dosage Modifications for Adverse Reactions
Grade 3*: Severe or medically significant but not immediately life-threatening; hospitalization or prolongation of hospitalization indicated; disabling; limiting self-care.
Grade 4*: Life-threatening consequences; urgent intervention indicated.

- Infusion-Related Reactions:
  - Grade 3 or 4: Permanently discontinue Niktimvo
- Elevation of ALT/AST (on day of dosing): ○ Grade 4: Permanently discontinue Niktimvo
- Elevation of CPK, amylase, or lipase:
  - Symptomatic ≥ Grade 3: Permanently discontinue Niktimvo

*Graded per National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE)

BACKGROUND AND OTHER CONSIDERATIONS

BACKGROUND:
Graft-versus-host disease (GVHD) occurs when immune cells transplanted from a non-identical donor (the graft) recognize the transplant recipient (the host) as foreign, thereby initiating an immune reaction that causes disease in the transplant recipient. Chronic graft-versus-host disease (cGVHD) is the major determinant of long-term outcome and quality of life following allogenic hematopoietic cell transplantation (HCT). Symptoms usually present within 3 years after allogeneic HCT and are often preceded by a history of acute GVHD. Manifestations of chronic GVHD may be restricted to a single organ or tissue or may be widespread. The primary manifestations of cGVHD are sclerotic cutaneous effects, dry oral mucosa, ulcerations and sclerosis of the gastrointestinal tract, and elevated serum bilirubin. Chronic GVHD can lead to debilitating consequences, e.g., joint contractures, loss of sight, end stage lung disease, or mortality resulting from profound chronic immune suppression leading to recurrent or life-threatening infections. Approximately half of patients with cGVHD become steroid refractory (SR), which greatly increases the risk of poor outcomes.

Niktimvo is a first-in-class agent to target CSF-1R, a cell surface protein that controls the survival and function of monocytes and macrophages, thereby reducing inflammation and fibrosis. Niktimvo is administered as an intravenous infusion over 30 minutes once every 2 weeks until disease progression or unacceptable toxicity. Niktimvo represents a new mechanism of action (MOA) for patients who fail to respond to other immunosuppressive therapies for cGVHD. Niktimvo will enter the cGVHD space as an IV therapy amid a primarily oral-therapy market. The efficacy of NIKTIMVO was evaluated in AGAVE-201 (NCT04710576), a randomized, open-label, multicenter study in adult and pediatric patients (n=79) with recurrent or refractory cGVHD who had received at least 2 lines of systemic therapy and required additional treatment. Treatment consisted of NIKTIMVO 0.3 mg/kg administered intravenously every 2 weeks until disease progression, lack of efficacy by 9 months, or unacceptable toxicity. Continued treatment with GVHD prophylaxis and standard care systemic cGVHD therapies were permitted as long as the patient had been on a stable dose for at least 2 weeks prior to study. Initiation of new systemic cGVHD therapy while on study was not permitted. The overall response rate was 74% (n=59) patients experiencing a response in first six cycles (3 months). The median time to first response was 1.5 months (range, 0.9 to 5.1 months). The median duration of response, calculated from first response to progression, death, or new systemic therapies for cGVHD, was 1.9 months (95% CI: 1.6, 3.5). In patients who achieved response, no death or new systemic therapy initiation occurred in 60% (95% CI: 43, 74) of patients for at least 12 months since response. The most common TRAEs included elevated levels of aspartate

aminotransferase (AST) (36%), blood creatine phosphokinase (CPK) (35%), lipase (29%), lactate dehydrogenase (27%), and alanine aminotransferase (ALT) (25%).

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:

All other uses of Niktimvo (axatilimab-csfr) are considered experimental/investigational and therefore, will follow Molina's Off- Label policy. Contraindications to Niktimvo (axatilimab-csfr) include: No labeled contraindications.

Exclusions/Discontinuation:

Based on its mechanism of action, Niktimvo may cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to the fetus. Advise females of reproductive potential to use effective contraception during treatment with Niktimvo and for 30 days after the last dose. Adverse reactions (i.e., infusion-related reactions or elevated lab values: ALT/AST, CPK, amylase, or lipase) may warrant discontinuation. (See Appendix)

OTHER SPECIAL CONSIDERATIONS: None

CODING/BILLING INFORMATION

CODING DISCLAIMER. *Codes listed in this policy are for reference purposes only and may not be allinclusive or applicable for every state or line of business. Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement. Listing of a service or device code in this policy does not guarantee coverage. Coverage is determined by the benefit document. Molina adheres to Current Procedural Terminology (CPT®), a registered trademark of the American Medical Association (AMA). All CPT codes and descriptions are copyrighted by the AMA; this information is included for informational purposes only. Providers and facilities are expected to utilize industrystandard coding practices for all submissions. Molina has the right to reject/deny the claim and recover claim payment(s) if it is determined it is not billed appropriately or not a covered benefit. Molina reserves the right to revise this policy as needed.*

HCPCS CODE	DESCRIPTION
J9038	Injection, axatilimab-csfr, 0.1 mg

AVAILABLE DOSAGE FORMS:

Niktimvo SOLN 9MG/0.18ML
Niktimvo SOLN 22MG/0.44ML

REFERENCES

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6. U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES. (2017). Common Terminology Criteria for Adverse Events (CTCAE) Common Terminology Criteria for Adverse Events (CTCAE) v5.0. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/CTCAE_v5_Quick_Reference_5x7.pdf

SUMMARY OF REVIEW/REVISIONS	DATE
NEW CRITERIA CREATION	Q2 2025