Corporate Medical Policy: Axatilimab-csfr (Niktimvo™) **"Notification"** **POLICY EFFECTIVE OCTOBER 1, 2025**

Restricted Product(s):

- axatilimab-csfr (Niktimvo™) intravenous infusion for administration by a healthcare professional

FDA Approved Use:

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

Criteria for Medical Necessity:

The restricted product(s) may be considered medically necessary when the following criteria are met:

Initial Criteria for Approval:

1. The patient is 6 years of age or older; **AND**
2. The patient weighs at least 40 kg; **AND**
3. The patient has a diagnosis of **chronic graft versus host disease (cGVHD)**; **AND**
4. The patient has presence of signs and symptoms of cGVHD (e.g., skin reactions, dry oral mucosa, gastrointestinal ulcerations, bronchiolar obstruction, etc.); **AND**
5. The patient has received allogeneic hematopoietic stem cell transplantation; **AND**
6. The patient has refractory or recurrent active disease despite receiving an adequate trial of at least two prior lines of systemic therapy for the treatment of cGVHD (e.g., corticosteroids, cyclosporine, tacrolimus, mycophenolate mofetil, methotrexate, sirolimus, everolimus, ruxolitinib, ibrutinib, belumosudil) **[medical record documentation required]**; **AND**
7. ONE of the following:
    a. The patient has tried and had an inadequate response to ibrutinib (Imbruvica) OR ruxolitinib (Jakafi) **[medical record documentation required]**; **OR**
    b. The patient has an intolerance or hypersensitivity to ibrutinib (Imbruvica) OR ruxolitinib (Jakafi) **[medical record documentation required]**; **OR**
    c. The patient has an FDA labeled contraindication to ibrutinib (Imbruvica) AND ruxolitinib (Jakafi) **[medical record documentation required]**; **AND**
8. ONE of the following:
    a. The patient has tried and had an inadequate response to belumosudil (Rezurock) **[medical record documentation required]**; **OR**

b. The patient has an intolerance or hypersensitivity to belumosudil (Rezurock) **[medical record documentation required]**; **OR**
c. The patient has an FDA labeled contraindication to belumosudil (Rezurock) **[medical record documentation required]**; **AND**

9. The prescriber is a specialist in the area of the patient's diagnosis (e.g., hematologist, oncologist) or has consulted with a specialist in the area of the patient's diagnosis; **AND**
10. The requested quantity does NOT exceed the maximum units allowed for the duration of approval (see table below); **AND**
11. For requests for injection or infusion administration of the requested medication in an **inpatient or outpatient hospital setting,** Site of Care Criteria applies (outlined below)*

Duration of Approval: 180 days (6 months)

Continuation Criteria for Approval:

1. The patient was approved through Blue Cross NC initial criteria for approval; **OR**
2. The patient would have met initial criteria for approval at the time they started therapy; **AND**
3. The patient has demonstrated a positive clinical response to treatment with the requested agent (e.g., improvement in cGVHD symptoms) **[medical record documentation required]**; **AND**
4. The prescriber is a specialist in the area of the patient's diagnosis (e.g., hematologist, oncologist) or has consulted with a specialist in the area of the patient's diagnosis; **AND**
5. The requested quantity does NOT exceed the maximum units allowed for the duration of approval (see table below); **AND**
6. For requests for injection or infusion administration of the requested medication in an **inpatient or outpatient hospital setting,** Site of Care Criteria applies (outlined below)*

Duration of Approval: 365 days (1 year)

FDA Label Reference				
Medication	**Indication**	**Dosing*,^**	**HCPCS**	**Maximum Units***
axatilimab-csfr (Niktimvo™) intravenous (IV) infusion	Chronic graft-versus-host disease (cGVHD) in adult and pediatric patients weighing ≥ 40 kg, after failure of ≥ 2 prior lines of systemic therapy	IV: 0.3 mg/kg (maximum 35 mg) every 2 weeks in adult and pediatric patients weighing ≥ 40 kg, until progression or unacceptable toxicity	J9038	Initial: 4,550 Continuation: 9,100

***Maximum units allowed for duration of approval**

***Site of Care Medical Necessity Criteria**

1. For requests for injection or infusion administration in an **inpatient setting**, the injection or infusion may be given if the above medical necessity criteria are met AND the inpatient admission is NOT for the sole purpose of administering the injection or infusion; **OR**
2. For requests for injection or infusion administration in an **outpatient hospital setting**, the injection or infusion may be given if the above medical necessity criteria are met AND ONE of the following must be met:
    a. History of mild adverse events that have not been successfully managed through mild pre-medication (e.g., diphenhydramine, acetaminophen, steroids, fluids, etc.); **OR**
    b. Inability to physically and cognitively adhere to the treatment schedule and regimen complexity; **OR**
    c. New to therapy, defined as initial injection or infusion OR less than 3 months since initial injection or infusion; **OR**
    d. Re-initiation of therapy, defined as ONE of the following:
        i. First injection or infusion after 6 months of no injections or infusions for drugs with an approved dosing interval less than 6 months duration; **OR**
        ii. First injection or infusion after at least a 1-month gap in therapy outside of the approved dosing interval for drugs requiring every 6 months dosing duration; **OR**
    e. Requirement of a change in the requested restricted product formulation; **AND**
3. If the Site of Care Medical Necessity Criteria in #1 or #2 above are not met, the injection or infusion will be administered in a **home-based infusion** or physician office setting with or without supervision by a certified healthcare professional.

References: all information referenced is from FDA package insert unless otherwise noted below.

1. Lee SJ, Wolff D, Kitko C, et al. Measuring therapeutic response in chronic graft-versus-host disease. National Institutes of Health consensus development project on criteria for clinical trials in chronic graft-versus-host disease: IV. The 2014 Response Criteria Working Group report. *Biol Blood Marrow Transplant*. 2015 Jun;21(6):984-99.
2. National Comprehensive Cancer Network Clinical Practice Guidelines in Oncology (NCCN Guidelines). Hematopoietic Cell Transplantation (HCT), Version 2.2024. Revised August 30, 2024. Available at https://www.nccn.org/professionals/physician_gls/pdf/hct.pdf. Last accessed February 19, 2025.
3. Wolff D, Cutler C, Lee SJ, et al. Safety and efficacy of axatilimab at 3 different doses in patients with chronic graft-versus-host disease (AGAVE-201). *Blood*. 2023;142:(Supplement 1):1.

Policy Implementation/Update Information: Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q2 annually.

October 2025: Criteria change: Added requirement for trial and failure of belumosudil (Rezurock). **Policy notification given 8/1/2025 for effective date 10/1/2025**.
June 2025: Criteria change: Removed requirement that Niktimvo not be used in combination with other cGVHD therapies.
April 2025: Coding change: Added HCPCS code J9038 to dosing reference table effective 4/1/2025; deleted C9399, J3490, J3590, and J9999 termed 3/31/2025. Adjusted maximum units according to coding unit definition for clarity.
February 2025: Original medical policy criteria issued.