# Niktimvo™ (Axatilimab-Csfr)

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**Community Plan Policy**

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## Coverage Rationale

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**Niktimvo (axatilimab-csfr) is proven for the treatment of chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy. Niktimvo is medically necessary for the treatment of chronic graft-versus-host disease (cGVHD) in patients who meet all of the following criteria:**

- For **initial therapy**, **all** of the following:
  - Diagnosis of chronic graft-versus-host disease (cGVHD) as defined by **all** of the following:
    - Patient has undergone allogenic hematopoietic cell transplantation (HCT); **and**
    - Clinical symptoms and signs that are components of the National Institutes of Health (NIH) consensus criteria for diagnosis of cGVHD; **and**
    - cGVHD is classified as moderate or severe based on grading scale (e.g., NIH disease grade, CIBMTR score)

    **and**
  - Patient has history of failure with **both** of the following:
    - Corticosteroid (e.g., prednisone, methylprednisolone); **and**
    - **One** of the following:
      - Bortezomib; **or**
      - Imbruvica (ibrutinib); **or**
      - Immunosuppressant agent (e.g., cyclosporine, mycophenolate, tacrolimus); **or**
      - Jakafi (ruxolitinib); **or**
      - Rezurock (belumosudil); **or**
      - Rituximab

    **and**
  - Patient weighs at least 40 kg; **and**
  - Prescribed by or in consultation with a transplant specialist; **and**
  - Niktimvo dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
  - Authorization will be issued for no more than 12 months
- For **continuation of therapy**, **all** of the following:
  - Documentation of a positive clinical response to Niktimvo therapy; **and**
  - Prescribed by or in consultation with a transplant specialist; **and**
  - Niktimvo dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
  - Reauthorization will be issued for no more than 12 months

**Niktimvo (axatilimab-csfr) is unproven and not medically necessary for prevention and/or treatment for all other indications, including but not limited to acute graft-versus-host disease (aGVHD), due to insufficient evidence of efficacy.**

## Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

| HCPCS Code | Description |
|---|---|
| J9038 | Injection, axatilimab-csfr, 0.1 mg |

| Diagnosis Code | Description |
|---|---|
| D89.811 | Chronic graft-versus-host disease |
| D89.812 | Acute on chronic graft-versus-host disease |

## Background

Graft-versus-host disease (GVHD) is a syndrome of variable clinical features that can develop after allogeneic hematopoietic cell transplant (HCT), whereby immune cells from a non-identical donor (i.e., the graft) initiate an immune response targeting a transplant recipient (i.e., the host).[4] There are two types of GVHD, acute and chronic, as well as an overlap syndrome with combined features of both types.[2] GVHD is characterized as a multisystem disorder that is delineated according to their clinical signs and symptoms based on the consensus criteria put forth by the National Institutes of Health (NIH).[2] Chronic graft-versus-host disease (cGVHD) is not a chronological extension following acute graft-versus-host disease (aGVHD), but rather a distinct syndrome which is independent of aGVHD.[4]

The clinical manifestations of cGVHD resembles autoimmune and other immunologic disorders that may be widespread or contained to a single organ or site. Nearly any body organ can be involved in cGVHD, while skin involvement is the primary clinical manifestation.[5] Other common affected organs and/or sites are the mouth, liver, lung, eye, and gastrointestinal tract.[4] cGVHD is one of the leading causes of morbidity and mortality after allogeneic HCT, which is directly correlated with the severity of the disease. More than one-third of patients who undergo allogeneic HCT require systemic treatment for severe cGVHD.[13]

Assessment of cGVHD includes identifying affected organ systems and the extent and severity of involvement in order properly determine prognosis and guide management.[4] Initial management of cGVHD is determined by the classification of the severity of cGVHD; mild, moderate, or severe. The treatment goals are to produce a sustained reduction or elimination of symptom burden, disability, and disease activity, while minimizing treatment-related adverse effects and while ultimately withdrawing therapy without a recurrence of cGVHD.[4]

## Benefit Considerations

Some Certificates of Coverage allow for coverage of experimental/investigational/unproven treatments for life-threatening illnesses when certain conditions are met. The member specific benefit plan document must be consulted to make coverage decisions for this service. Some states mandate benefit coverage for off-label use of medications for some diagnoses or under some circumstances when certain conditions are met. Where such mandates apply, they supersede language in the benefit document or in the medical or drug policy.

## Clinical Evidence

The efficacy of Niktimvo was established in AGAVE-201, a randomized, open-label study in 79 adult and pediatric patients with recurrent or refractory cGVHD who had received at least 2 lines of systemic therapy and required additional treatment. Treatment consisted of Niktimvo administered intravenously every 2 weeks until disease progression, lack of efficacy by 9 months, or unacceptable toxicity. The primary endpoint was overall response rate (ORR) through cycle 7 day 1, where overall response included complete response or partial response according to the 2014 NIH Consensus

Development Project on Response Criteria. Among patients who received Niktimvo at the approved dose of 0.3 mg/kg every two weeks (n = 79), 75% achieved an overall response rate (ORR) within the first six months of treatment, with a median time to response of 1.5 months. Among those who achieved a response, 60% maintained a response at 12 months (measured from first response to new systemic therapy or death, based on the Kaplan Meier estimate). The trial also demonstrated an exploratory endpoint, having a majority (56%) of patients showing a ≥ 7-point improvement in the modified Lee Symptom Scale (mLSS) score. 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).

## U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

Niktimvo is a colony stimulating factor-1 receptor (CSF-1R)-blocking antibody indicated for the 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.

## Centers for Medicare and Medicaid Services (CMS)

Medicare does not have a National Coverage Determination (NCD) for Niktimvo™ (axatilimab-csfr). Local Coverage Determinations (LCDs)/Local Coverage Articles (LCAs) do not exist.

In general, Medicare covers outpatient (Part B) drugs that are furnished "incident to" a physician's service provided that the drugs are not usually self-administered by the patients who take them. Refer to the Medicare Benefit Policy Manual, Chapter 15, §50 - Drugs and Biologicals. (Accessed February 10, 2025)

## References


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13. Arora M, Cutler CS, Jagasia MH, et al. Late Acute and Chronic Graft-versus-Host Disease after Allogeneic Hematopoietic Cell Transplantation. Biol Blood Marrow Transplant 2016; 22:449.

# Policy History/Revision Information

| Date | Summary of Changes |
|---|---|
| 09/01/2025 | • Corrected formatting error in *References* section |
| 07/01/2025 | **Coverage Rationale**<br>• Removed reference link to the Medical Benefit Drug Policy titled *Review at Launch for New to Market Medications*<br>**Supporting Information**<br>• Updated *Benefit Considerations* section to reflect the most current information<br>• Archived previous policy version 2025D0135A |

# Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting UnitedHealthcare standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, please check the member specific benefit plan document and any applicable federal or state mandates. UnitedHealthcare reserves the right to modify its Policies and Guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

This Medical Benefit Drug Policy may also be applied to Medicare Advantage plans in certain instances. In the absence of a Medicare National Coverage Determination (NCD), Local Coverage Determination (LCD), or other Medicare coverage guidance, CMS allows a Medicare Advantage Organization (MAO) to create its own coverage determinations, using objective evidence-based rationale relying on authoritative evidence (Medicare IOM Pub. No. 100-16, Ch. 4, §90.5).

UnitedHealthcare may also use tools developed by third parties, such as the InterQual® criteria, to assist us in administering health benefits. UnitedHealthcare Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.