Oscar Clinical Guideline: Niktimvo (axatilimab) (PG252, Ver. 1)

# Niktimvo (axatilimab)

**Disclaimer**

*Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.*

*Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.*

## Summary

Chronic graft-versus-host disease (cGVHD) is a serious complication of allogeneic hematopoietic stem cell transplantation, occurring in 30-70% of patients. It is a complex, multisystem disorder characterized by immune dysregulation, leading to fibrosis and organ dysfunction. cGVHD can affect various organs, including the skin, eyes, mouth, lungs, gastrointestinal tract, liver, and musculoskeletal system.

Treatment options for cGVHD include systemic corticosteroids as first-line therapy, followed by various immunosuppressive agents such as calcineurin inhibitors, mycophenolate mofetil, sirolimus, and targeted therapies like ibrutinib and ruxolitinib. Despite these options, many patients experience inadequate response or intolerance to multiple lines of therapy.

Niktimvo (axatilimab) is a colony stimulating factor-1 receptor (CSF-1R)-blocking antibody indicated for the treatment of cGVHD in adult and pediatric patients weighing at least 40 kg after failure of at least two prior lines of systemic therapy.

## Definitions

"**Allogeneic hematopoietic stem cell transplantation**" is a procedure in which a patient receives blood-forming stem cells from a genetically similar, but not identical, donor.

"**Chronic graft-versus-host disease (cGVHD)**" is a complex, multisystem disorder that occurs following allogeneic hematopoietic stem cell transplantation, characterized by immune dysregulation and fibrosis affecting various organs.

"**Steroid-refractory cGVHD**" refers to disease that fails to improve despite treatment with prednisone at ≥1 mg/kg/day for at least 1 week or persists without improvement despite continued treatment with prednisone at ≥0.5 mg/kg/day or 1 mg/kg every other day for at least 4 weeks.

## Medical Necessity Criteria for Initial Authorization

The Plan considers **Niktimvo (axatilimab)** medically necessary when **ALL** of the following criteria are met:

1. The medication is prescribed by or in consultation with a hematologist, oncologist, or transplant specialist; ***AND***
2. The member is 12 years of age or older **AND** weighs at least 40 kg; ***AND***
3. The member has a diagnosis of chronic graft-versus-host disease (cGVHD); ***AND***
4. The member meets **ALL** of the following criteria:
    a. Has chronic graft-versus-host disease (cGVHD) that is steroid-refractory, defined as:
        i. Lack of response or disease progression after administration of prednisone *(or equivalent dose of another corticosteroid [1])* at ≥1 mg/kg/day for at least 1 week; ***or***

        *[1]e.g., for dexamethasone, an equivalent dose is considered to be ≥0.15 mg/kg/day.*
        ii. Disease persistence without improvement despite continued treatment with prednisone *(or equivalent dose of another corticosteroid [2])* at ≥0.5 mg/kg/day or 1 mg/kg every other day for at least 4 weeks; ***and***

        *[2]e.g., for dexamethasone, an equivalent dose is considered to be ≥0.075 mg/kg/day or ≥0.15 mg/kg every other day.*
    b. Has failed at least **TWO** prior lines of systemic therapy for cGVHD, including:
        i. Ruxolitinib (Jakafi); ***and***
        ii. One additional systemic therapy for cGVHD. Prior therapies may include, but are not limited to:

1. Abatacept; ***and/or***
2. Belumosudil (Rezurock); ***and/or***
3. Calcineurin inhibitors (e.g., tacrolimus, cyclosporine); ***and/or***
4. Extracorporeal photopheresis; ***and/or***
5. Hydroxychloroquine (Plaquenil); ***and/or***
6. Ibrutinib (Imbruvica); ***and/or***
7. Imatinib (Gleevec); ***and/or***
8. Interleukin-2 (IL-2), such as basiliximab (Simulect); ***and/or***
9. Low-dose methotrexate; ***and/or***
10. mTOR inhibitors (e.g., sirolimus, everolimus); ***and/or***
11. Mycophenolate mofetil; ***and/or***
12. Pentostatin (Nipent); ***and/or***
13. Rituximab (Rituxan); ***and***

c. Will receive Niktimvo (axatilimab) in conjunction with systemic corticosteroids; ***AND***

5. Documentation of specific therapies tried, duration of treatment, and reason for discontinuation (e.g., lack of efficacy, adverse effects) is provided.

**If the above prior authorization criteria are met, the requested product will be authorized for 6-months.**

## Medical Necessity Criteria for Reauthorization

Reauthorization for 12-months will be granted if the member has recent (within the last 3-months) clinical chart documentation demonstrating **ALL** of the following criteria:

1. The requested medication is prescribed by or in consultation with a hematologist, oncologist, or transplant specialist***; AND***
2. The member has experienced clinical benefit from therapy as evidenced by at least **ONE** of the following:
    a. Improvement in cGVHD symptoms***; or***
    b. Reduction in immunosuppressive medication requirements***; or***
    c. Improvement in performance status.

## Experimental or Investigational / Not Medically Necessary

Niktimvo (axatilimab) for any other indication or use is considered not medically necessary by the Plan, as it is deemed to be experimental, investigational, or unproven. Non-covered indications include, but are not limited to, the following:

- The treatment of acute graft-versus-host disease.
- The treatment of cGVHD in patients weighing less than 40 kg.
- The treatment of cGVHD as first- or second-line therapy.
- The treatment of any condition other than cGVHD.

## Applicable Billing Codes (HCPCS/CPT Codes)

| ***Service(s) name*** | |
|---|---|
| **CPT/HCPCS Codes considered medically necessary if criteria are met:** | |
| *Code* | *Description* |
| 96365 | Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); initial, up to 1 hour |
| 96366 | Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); each additional hour (List separately in addition to code for primary procedure) |
| C9399 | Unclassified drugs or biologicals |
| J3590 | Unclassified biologics |
| **ICD-10 codes considered medically necessary if criteria are met:** | |
| *Code* | *Description* |
| D89.811 | Chronic graft-versus-host disease |
| D89.812 | Acute on chronic graft-versus-host disease |
| D89.813 | Graft-versus-host disease, unspecified |
| T86.09 | Other complications of bone marrow transplant |

## References


1. National Comprehensive Cancer Network. NCCN Clinical Practice Guidelines in Oncology: Hematopoietic Cell Transplantation. Version 2.2024. Published August 30, 2024. Accessed October 10, 2024. https://www.nccn.org/.
2. Niktimvo (axatilimab) [prescribing information]. Wilmington, DE: Incyte Corporation; August 2024.
3. Penack O, Marchetti M, Ruutu T, Aljurf M, Bacigalupo A, Bonifazi F, Ciceri F, Cornelissen J, Malladi R, Duarte RF, Giebel S, Greinix H, Holler E, Lawitschka A, Mielke S, Mohty M, Arat M, Nagler A, Passweg J, Schoemans H, Socié G, Solano C, Vrhovac R, Zeiser R, Kröger N, Basak GW. Prophylaxis and management of graft versus host disease after stem-cell transplantation for haematological malignancies: updated consensus recommendations of the European Society for Blood and Marrow Transplantation. Lancet Haematol. 2020 Feb;7(2):e157-e167. doi: 10.1016/S2352-3026(19)30256-X. PMID: 32004485.
4. Zeiser R, Polverelli N, Ram R, Hashmi SK, Chakraverty R, Middeke JM, Musso M, Giebel S, Uzay A, Langmuir P, Hollaender N, Gowda M, Stefanelli T, Lee SJ, Teshima T, Locatelli F; REACH3 Investigators. Ruxolitinib for Glucocorticoid-Refractory Chronic Graft-versus-Host Disease. N Engl J Med. 2021 Jul 15;385(3):228-238. doi: 10.1056/NEJMoa2033122. PMID: 34260836.


## Clinical Guideline Revision / History Information

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| Original Date: 10/29/2024 |
| Reviewed/Revised: |