

## Clinical Policy: Axatilimab-csfr (Niktimvo)

Reference Number: PA.CP.PHAR.691

Effective Date: 11/2024

Last Review Date: 10/2025

### Description

Axatilimab-csfr (Niktimvo™) is a colony stimulating factor-1 receptor (CSF-1R)-blocking antibody.

### FDA Approved Indication(s)

Niktimvo is 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.

### Policy/Criteria

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

It is the policy of PA Health & Wellness® that Niktimvo is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

##### A. Chronic Graft-Versus-Host Disease (must meet all):

1. Diagnosis of cGVHD post hematopoietic cell transplantation;
2. Prescribed by or in consultation with an oncologist, hematologist, or bone marrow transplant specialist;
3. Weight  $\geq$  40 kg;
4. Failure of a systemic corticosteroid (see Appendix B for examples) at up to maximally indicated doses, unless contraindicated or clinically significant adverse effects are experienced;
5. Failure of a systemic non-steroidal immunosuppressant\* (see Appendix B for examples) at up to maximally indicated doses, unless contraindicated or clinically significant adverse effects are experienced; \*Prior authorization may be required
6. Niktimvo is not prescribed concurrently with Jakafi®, Imbruvica®, or Rezurock®;
7. Request meets one of the following (a or b):
  - a. Dose does not exceed 0.3 mg/kg (up to maximum of 35 mg) every 2 weeks;
  - b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

**Approval duration: 12 months**

##### B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

## II. Continued Therapy

### A. Chronic Graft-Versus-Host Disease (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy;
3. Niktimvo is not prescribed concurrently with Jakafi, Imbruvica, or Rezurock;
4. If request is for a dose increase, request meets one of the following (a or b):
  - a. New dose does not exceed 0.3 mg/kg (up to maximum of 35 mg) every 2 weeks;
  - b. New dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

**Approval duration: 12 months**

### B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.

**Approval duration: Duration of request or 12 months (whichever is less); or**

2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

## III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

## IV. Appendices/General Information

### *Appendix A: Abbreviation/Acronym Key*

cGVHD: chronic graft-versus-host disease

CSF-1R: colony stimulating factor-1 receptor

FDA: Food and Drug Administration

### *Appendix B: Therapeutic Alternatives*

This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Systemic corticosteroids (e.g., methylprednisolone, prednisone)	Varies	Varies
Jakafi (ruxolitinib)	10 mg PO BID	20 mg/day*
Imbruvica (ibrutinib)	420 mg PO QD	420 mg/day
Rezurock (belumosudil)	200 mg PO QD	200 mg/day
Orencia <sup>®</sup> (abatacept) <sup>†</sup>		1 g/dose

Campath <sup>®</sup> (alemtuzumab) †	10 mg SC QD for 3 days or 3 mg IV TIW, then 10 mg IV weekly	See regimen
tacrolimus (Prograf <sup>®</sup> ) †	0.15 mg/kg PO BID	Based on serum concentrations
cyclosporine (Gengraf <sup>®</sup> , Neoral <sup>®</sup> , Sandimmune <sup>®</sup> ) †	6 mg/kg PO BID	Based on serum concentrations
Enbrel <sup>®</sup> (etanercept) †	0.4 mg/kg SC TIW	50 mg/week
imatinib (Gleevec <sup>®</sup> ) †	100 mg PO QD	400 mg/day
Proleukin <sup>®</sup> (aldeskeukin)		720,000 units/kg every 8 hours for up to 12 doses
sirolimus (Rapamune <sup>®</sup> ) †	0.25 to 0.5 mg PO QD	40 mg/day*
mycophenolate mofetil (Cellcept <sup>®</sup> ) †	240 mg PO QID or 1 g PO BID	2 g/day*
Nipent (pentostatin) †	4 mg/m <sup>2</sup> IV every 2 weeks	4 mg/m <sup>2</sup> /2 weeks*
rituximab (Riabni <sup>®</sup> , Rituxan <sup>®</sup> , Ruxience <sup>®</sup> , Truxima <sup>®</sup> ) †	375 mg/m <sup>2</sup> IV weekly	1,000 mg/week*

Therapeutic alternatives are listed as Brand name<sup>®</sup> (generic) when the drug is available by brand name only and generic (Brand name<sup>®</sup>) when the drug is available by both brand and generic.

\*Maximum dose of the drug, not indication specific

†Off-label

#### Appendix C: Contraindications/Boxed Warnings

None reported

### V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
cGVHD	0.3 mg/kg (up to a maximum of 35 mg) IV infusion every 2 weeks	35 mg/2 weeks

### VI. Product Availability

Single-dose vials: 9 mg/0.18 mL, 22 mg/0.44 mL, 50 mg/mL

### VII. References

1. Niktimvo Prescribing Information. Wilmington, DE: Incyte Corporation; August 2024. Available at: [www.niktimvohcp.com](http://www.niktimvohcp.com). Accessed July 23, 2025.
2. Clinical Pharmacology [database online]. Tampa, FL: Elsevier, Inc.; 2024. Available at: <http://www.clinicalkey.com/pharmacology/>. Accessed July 23, 2025.
3. National Comprehensive Cancer Network. Hematopoietic Cell Transplantation (HCT) 2.2025. Available at: [https://www.nccn.org/professionals/physician\\_gls/pdf/hct.pdf](https://www.nccn.org/professionals/physician_gls/pdf/hct.pdf). Accessed July 23, 2025.
4. Wolff D, Cutler C, Lee SJ, et al; AGAVE-201 Investigators. Axatilimab in recurrent or refractory chronic graft-versus-host disease. *N Engl J Med*. 2024 Sep 19;391(11):1002-1014. doi: 10.1056/NEJMoa2401537.

**Coding Implications**

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J9038	Injection, axatilimab-csfr, 0.1 mg

Reviews, Revisions, and Approvals	Date
Policy created	10/2024
4Q 2025 annual review: clarified systemic immunosuppressant as non-steroidal; extended initial approval duration from 6 months to 12 months for this maintenance medication for a chronic condition; RT4: added new dosage strengths of 9 mg/0.18 mL and 22 mg/0.44 mL; HCPCS code added [J9038], removed codes [J3590, C9399]; references reviewed and updated.	10/2025