

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Graft-Versus-Host Disease – Niktimvo Utilization Management Medical Policy

- Niktimvo™ (axatilimab-csfr intravenous infusion – Incyte/Syndax)

REVIEW DATE: 5/07/2025

OVERVIEW

Niktimvo, a colony stimulating factor-1 receptor-blocking antibody, is indicated for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.

Guidelines

Niktimvo has been addressed in the National Comprehensive Cancer Network Hematopoietic Cell Transplantation guidelines (version 1.2025 – February 28, 2025). Options for first-line therapy for chronic GVHD including restarting, continuing, or escalating the original immunosuppressive agent(s) and/or administering systemic corticosteroids (0.5 to 1 mg/kg day of methylprednisolone or prednisone dose equivalent). Among the agents FDA-approved for use in chronic GVHD, Jakafi® (ruxolitinib tablets) is the only agent given a category 1 recommendation for chronic GVHD. Niktimvo, Rezurock® (belumosudil tablets), and Imbruvica® (ibrutinib tablets, capsules, and oral suspension) each have a category 2A recommendation. The guidelines cite that each of these FDA-approved agents should be used following failure of one or two lines of systemic therapy (depending on the agent). Other medication alternatives include Orencia® (abatacept intravenous [IV] infusion and subcutaneous [SC] injection), Lemtrada® (alemtuzumab IV infusion), calcineurin inhibitors (e.g., tacrolimus, cyclosporine), Enbrel® (etanercept SC injection), extracorporeal photopheresis, hydroxychloroquine, imatinib, Proleukin® (aldesleukin IV infusion and SC injection), low-dose methotrexate, mammalian target of rapamycin inhibitors (e.g., sirolimus), mycophenolate mofetil, pentostatin, and rituximab.

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Niktimvo. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication(s). Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Niktimvo is recommended in those who meet the following criteria:

FDA-Approved Indication

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- 1. Graft-Versus-Host Disease.** Approve for 1 year if the patient meets ONE of the following (A or B):
 - A) Initial Therapy.** Approve if the patient meets ALL of the following (i, ii, and iii):
 - i.** Patient is ≥ 40 kg; AND
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- ii. Patient has chronic graft-versus-host disease; AND
- iii. Patient has tried at least two systemic medications for chronic graft-versus-host disease; OR
Note: Examples of systemic medications include Jakafi (ruxolitinib tablets), Rezurock (belumosudil tablets), Imbruvica (ibrutinib tablets, capsules, and oral suspension), imatinib, hydroxychloroquine, methotrexate, rituximab, pentostatin, interleukin-2 (e.g., Proleukin [aldesleukin intravenous infusion]), methylprednisolone, cyclosporine, tacrolimus, sirolimus, an etanercept product, and mycophenolate mofetil.

B) Patient Currently Receiving Niktimvo. Approve if according to the prescriber, the patient demonstrates a beneficial clinical response.

Note: Examples of a beneficial response include a reduction in corticosteroid dose, disease stabilization, and/or symptomatic improvement.

Dosing. Approve 0.3 mg/kg (up to a maximum dose of 35 mg) given as an intravenous infusion once every 2 weeks.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Niktimvo is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Niktimvo™ intravenous infusion [prescribing information]. Wilmington, DE and Waltham, MA: Incyte/Syndax; January 2025.
2. The NCCN Hematopoietic Cell Transplantation Clinical Practice Guidelines in Oncology (version 1.2025 – February 28, 2025). © 2025 National Comprehensive Cancer Network. Available at: <http://www.nccn.org>. Accessed on March 19, 2025.

HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	10/23/2024
Early Annual Revision	Graft-Versus-Host Disease was added to the header. For Graft-Versus-Host Disease , the criteria were divided into “Initial Therapy” and “Patient Currently Receiving Niktimvo”. For initial therapy, for the requirement that a patient tried two systemic treatments, the descriptor “conventional” was removed and the word “therapies” was changed to “medications”. Also, an etanercept product was listed in the Note that cites examples of systemic therapy for chronic graft-versus-host disease. For a patient currently receiving Niktimvo, an approval is given if according to the prescriber, the patient demonstrates a beneficial clinical response. A Note was added that a beneficial response can include a reduction in corticosteroid dose, disease stabilization, and/or symptomatic improvement.	05/07/2025