

DRUG POLICY

Niktimvo™ (axatilimab-csfr)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

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 for Initial Approval

Chronic graft-versus-host disease (cGVHD)

Authorization of 12 months may be granted for treatment of cGVHD when ALL of the following criteria are met:

1. The member has had at least two prior lines of systemic therapy that failed to produce the desired response
2. The member weighs at least 40 kg

Continuation of Therapy

Chronic graft-versus-host disease (cGVHD)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for cGVHD when there is no evidence of unacceptable toxicity or disease progression while on the current regimen.

Other

Niktimvo (axatilimab-csfr) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

CLINICAL RATIONALE

Graft-versus-host disease (GVHD) is a condition that can develop after an allogeneic hematopoietic cell transplant (aHCT), in which the donor lymphocytes recognize the recipient's tissues as foreign, resulting in immune-mediated cellular injury of several bodily organs, such as the skin, gastrointestinal (GI) tract, and the liver. GVHD is a major complication of allogeneic HCT and is associated with significant morbidities and mortality. GVHD can be classified as acute or chronic GVHD. Chronic GVHD usually develops within the first year after HCT, but can also develop many years later and is associated with features such as fasciitis, lichen sclerosis-like skin changes, and esophageal webbing.

First-line therapy for the treatment of chronic GVHD includes continuation or resumption of original immunosuppressive agent with/or systemic corticosteroids. About 50% of patients will not respond and be classified as having steroid-refractory disease. FDA approved drugs for cGVHD include Ibruvica (ibrutinib), Jakafi (ruxolitinb), Rezurock (belumosudil) and Niktimvo (axatilimab-csfr). As alternative agents for steroid-refractory cGVHD, the NCCN Guidelines also recommend abatacept, alemtuzumab, calcineurin inhibitors, entanercept, extracorporeal photopheresis, hydroxychloroquine, imatinib, interleukin-2, methotrexate, mTOR inhibitors, mycophenolate mofetil, pentostatin, and rituximab.

Niktimvo (axatilimab-csfr) was evaluated in a phase 2, open-label, randomized, multicenter trial (AGAVE-201). The trial enrolled 241 patients who had a diagnosis of cGVHD and had failed at least 2 prior lines of systemic therapy with patients receiving a median of four prior lines of systemic therapy for cGVHD. Concomitant use of corticosteroids, calcineurin inhibitors and mTOR inhibitors was allowed. Patients were assigned to receive axatilimab-csfr 0.3 mg/kg every 2 weeks, 1.0 mg/kg every 2 weeks, or 3.0 mg/kg every 4 weeks with a primary endpoint of overall response rate (complete or partial) by week 24 and the key secondary endpoint was the decrease in patient-reported symptom burden as assessed by the modified Lee Symptom Scale. Note that the FDA-approved dose is 0.3 mg/kg every 2 weeks.

	Niktimvo 0.3 mg/kg Intravenous every 2 weeks (n=79)
Overall Response Rate, %	75 (64 to 84)
Complete Response, %	0
Partial Response, %	75
Clinical benefit: ≥7-point reduction in modified Lee Symptom Scale score, %	56 (44 to 67)

Among participants in the AGAVE-201 trial who received the FDA-approved dose, the incidence of treatment-emergent adverse events (TEAEs) was 96%, drug discontinuation due to adverse event occurred in 6%, Grade ≥3 adverse events occurred in 49% and one patient experienced a fatal adverse event. The most common reactions were infection, headache, diarrhea, laboratory abnormalities, infusion related reactions, and hypertension.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- J9038 – Injection, axatilimab-csfr, 0.1 mg

REFERENCES

Niktimvo [package insert]. Wilmington, DE: Incyte Corporation; January 2025.

The NCCN Drugs & Biologics Compendium® ©2025 National Comprehensive Cancer Network, Inc.
Available at: <http://www.nccn.org>. Accessed January 7, 2025.

POLICY HISTORY

Policy #: 05.05.83

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