Clinical Policy: Naxitamab-gqgk (Danyelza)

Reference Number: PA.CP.PHAR.523
Effective Date: 02/2022
Last Review Date: 01/2026

Description

Naxitamab-gqgk (Danyelza®) is a glycolipid disialoganglioside (GD2)-binding recombinant humanized monoclonal IgG1 antibody.

FDA Approved Indication(s)

Danyelza is indicated, in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. *

**This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).*

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 Danyelza is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Neuroblastoma (must meet all)**:**

1. Diagnosis of high-risk neuroblastoma;
2. Disease is relapsed, refractory or progressive;
3. Disease is occurring in the bone or bone marrow;
4. Prescribed by or in consultation with an oncologist or hematologist;
5. Age ≥ 1 year;
6. Prescribed in one of the following ways (a or b)
   a. In combination with GM-CSF (e.g., Leukine®);*
   b. In combination with GM-CSF, Temodar®*, and irinotecan;
   **Prior authorization may be required for Leukine and Temodar*
7. One of the following (a or b):
   a. Member has demonstrated a partial response, minor response, or stable disease to prior therapy (*see Appendix B for examples*);
   b. Member has progressive disease;
8. Request meets one of the following (a or b):
   a. Dose does not exceed 150 mg (4 vials) per day for 3 days of each 4-week treatment cycle;
   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. Neuroblastoma (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. If request is for a dose increase, request meets one of the following (a or b):
   a. New dose does not exceed 150 mg (4 vials) per day for 3 days of each 4- or 8-week treatment cycle;
   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 6 months (whichever is less); or
2. 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

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

COG: Children's Oncology Group
FDA: Food and Drug Administration
GD2: glycolipid disialoganglioside
INRG: International Neuroblastoma Risk Group
INRGSS: International Neuroblastoma Risk Group Staging System
INSS: International Neuroblastoma Staging System
GM-CSF: granulocyte-macrophage colony-stimulating factor

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
cisplatin, etoposide, vincristine, cyclophosphamide, doxorubicin, topotecan	Used in various combinations in variable dosing regimens	Varies
Unituxin® (dinutuximab), isotretinoin, GM-CSF	Used in various combinations in variable dosing regimens	Varies

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

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): history of hypersensitivity reaction to naxitamab-gqgk
- Boxed warning(s): serious infusion-related reactions and neurotoxicity

Appendix D: General Information

- Defining "high-risk" neuroblastoma: The Children's Oncology Group (COG) risk group system is using the International Neuroblastoma Risk Group Staging System (INRGSS), along with the major prognostic factors to place children into 3 different risk groups: low, intermediate, and high. High-risk neuroblastoma patients, per NCCN's COG-adapted risk classifier, are dependent on INRG tumor staging (L1, L2, M, MS), age at diagnosis, tumor MYCN amplification status, histopathology, status of segmental chromosome aberrations and DNA index (diploid or hyperdiploid).

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Neuroblastoma	3 mg/kg/day IV on Days 1, 3, and 5 of each 28-day treatment cycle. Treatment cycles are repeated every 4 weeks until complete response or partial response, followed by 5 additional cycles every 4 weeks. Subsequent cycles may be repeated every 8 weeks.	150 mg/day

VI. Product Availability

Injection solution in a single-dose vial: 40 mg/10 mL

VII. References

1. Danyelza Prescribing Information. New York, NY; August 2025. Available at: https://labeling.ymabs.com/danyelza. Accessed October 21, 2025.
2. American Cancer Society. Neuroblastoma. Last revised June 26, 2025. Available at: https://www.cancer.org/cancer/types/neuroblastoma.html. Accessed November 29, 2025.
3. National Comprehensive Cancer Network Drugs and Biologics Compendium. Available at: http://www.nccn.org/professionals/drug_compendium. Accessed November 29, 2025.

4. National Comprehensive Cancer Network. Neuroblastoma Version 1.2025. Available at: https://www.nccn.org/professionals/physician_gls/pdf/mds.pdf. Accessed November 29, 2025.

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
J9348	Injection, naxitamab-gqgk, 1 mg

Reviews, Revisions, and Approvals	Date
Policy created	01/2022
1Q 2023 annual review: no significant changes; references reviewed and updated.	01/2023
1Q 2024 annual review: no significant changes; references reviewed and updated.	01/2024
1Q 2025 annual review: no significant changes; references reviewed and updated.	01/2025
1Q 2026 annual review: added treatment combination option with GM-CSF, Temodar, and irinotecan per NCCN; revised Medicaid/HIM initial approval duration to 12 months; references reviewed and updated.	01/2026