Original Effective Date: 07/17/2025
Current Effective Date: 03/13/2026
Last P&T Approval/Version: 01/28/2026
Review Due By: 01/2027
Policy Number: C29408-A

Gomekli (mirdametinib)

PRODUCTS AFFECTED

Gomekli (mirdametinib)

COVERAGE POLICY

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Coverage Guideline must be read in its entirety to determine coverage eligibility, if any. This Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide Molina Healthcare complete medical rationale when requesting any exceptions to these guidelines.

Documentation Requirements:
Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive.

DIAGNOSIS:
Neurofibromatosis type 1 (NF1) plexiform neurofibromas (PN)

REQUIRED MEDICAL INFORMATION:
This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. If a drug within this policy receives an updated FDA label within the last 180 days, medical necessity for the member will be reviewed using the updated FDA label information along with state and federal requirements, benefit being administered and formulary preferencing. Coverage will be determined on a case-by-case basis until the criteria can be updated through Molina Healthcare, Inc. clinical governance. Additional information may be required on a case-by-case basis to allow for adequate review. When the requested drug product for coverage is dosed by weight, body surface area or other member specific measurement, this data element is required as part of the medical necessity review. The Pharmacy and Therapeutics Committee has determined that the drug benefit shall be a mandatory generic and

that generic drugs will be dispensed whenever available.

A. NEUROFIBROMATOSIS TYPE 1 PLEXIFORM NEUROFIBROMAS:
1. Documented diagnosis of Neurofibromatosis type 1 (NF1) plexiform neurofibromas (PN) AND
2. Documentation that complete resection of PN is not considered to be feasible without substantial risk or morbidity (e.g., due to encasement of, or close proximity to, vital structures, invasiveness, or high vascularity of the PN) AND
3. Documentation that member is symptomatic (i.e., experiencing pain, motor dysfunction, and visual loss) AND
4. Documentation member's BSA is at least 0.40 m2

CONTINUATION OF THERAPY:
A. NEUROFIBROMATOSIS TYPE 1 PLEXIFORM NEUROFIBROMAS:
1. Adherence to therapy at least 85% of the time as verified by the prescriber or member medication fill history (review Rx history for compliance) unless therapy held for toxicity AND
2. Documentation of positive clinical response as demonstrated by no evidence of disease progression or unacceptable toxicity (See Appendix)

DURATION OF APPROVAL:
Initial authorization: 12 months, Continuation of Therapy: 12 months

PRESCRIBER REQUIREMENTS:
Prescribed by or in consultation with an oncologist or a neurologist [If prescribed in consultation, consultation notes must be submitted with initial request and reauthorization requests]

AGE RESTRICTIONS:
2 years of age and older

QUANTITY:
2 mg/m2 twice daily - See chart

Body Surface Area*	Recommended Dosage
0.40 to 0.69 m2	1 mg twice daily
0.70 to 1.04 m2	2 mg twice daily
1.05 to 1.49 m2	3 mg twice daily
≥1.50 m2	4 mg twice daily

*The recommended dosage for patients with a BSA less than 0.40 m2 has not been established.

Maximum Quantity Limits: 4 mg twice daily

PLACE OF ADMINISTRATION:
The recommendation is that oral medications in this policy will be for pharmacy benefit coverage and patient self-administered.

DRUG INFORMATION

ROUTE OF ADMINISTRATION:
Oral

DRUG CLASS:
Antineoplastic - MEK Inhibitors

FDA-APPROVED USES
Indicated for the treatment of adult and pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic plexiform neurofibromas (PN) not amenable to complete resection.

COMPENDIAL APPROVED OFF-LABELED USES:
Primary Spinal Cord Tumors (NCCN Central Nervous System Cancers Primary Spinal Cord Tumors: Systemic Therapy PSCT-A)

APPENDIX

APPENDIX:
Adverse Reactions requiring permanent discontinuation of Gomekli:

- Left Ventricular Dysfunction: Any absolute decrease in LVEF 20% or greater from baseline
- Ocular Toxicity: Retinal vein occlusion (RVO)

Monitor for severe skin rashes. Withhold, reduce the dose, or permanently discontinue Gomekli based on severity.

BACKGROUND AND OTHER CONSIDERATIONS

BACKGROUND:
Neurofibromatosis type 1 (NF1) is a rare, progressive genetic disorder caused by mutations in the NF1 gene, with an incidence of approximately 1 in 2,500 births. A hallmark of NF1 is the development of plexiform neurofibromas (PN), benign nerve sheath tumors that are often inoperable and can cause pain, disfigurement, and functional impairment.

Historically, treatment options for symptomatic, inoperable PN have been limited. Surgical resection is often not feasible due to the tumor's location and diffuse growth pattern. Recently, Koselugo (selumetinib), an oral mitogen-activated protein kinase kinases 1 and 2 (MEK1/2) inhibitor, was approved for the treatment of symptomatic, inoperable NF1-PN in pediatric patients (from 2 to 17 years of age).

Gomekli (mirdametinib), an oral MEK1/2 inhibitor, received FDA approval in February 2025 for the treatment of adults and pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable PN. Approval was based on the Phase 2 ReNeu trial, which demonstrated an objective response rate (≥20% reduction in PN volume) in a significant proportion of patients, with improvements in pain, function, and quality of life. The safety profile was manageable and consistent with other MEK inhibitors.

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:

All other uses of Gomekli (mirdametinib) are considered experimental/investigational and therefore, will follow Molina's Off-Label policy. Contraindications to Gomekli (mirdametinib) include: no labeled contraindications.

Exclusions/Discontinuation:

Adverse Reactions requiring permanent discontinuation of Gomekli:

- Left Ventricular Dysfunction: Any absolute decrease in LVEF 20% or greater from baseline
- Ocular Toxicity: Retinal vein occlusion (RVO)
- Monitor for severe skin rashes. Withhold, reduce the dose, or permanently discontinue Gomekli based on severity.

Verify the pregnancy status of females of reproductive potential prior to the initiation of Gomekli. Advise females of reproductive potential to use effective contraception during treatment with Gomekli and for 6 weeks after the last dose. Advise males with female partners of reproductive potential to use effective contraception during treatment with Gomekli and for 3 months after the last dose.

Women should be advised not to breastfeed during treatment with Gomekli and for 1 week after the last dose because of the potential for adverse reactions in breastfed children.

OTHER SPECIAL CONSIDERATIONS:

None

CODING/BILLING INFORMATION

CODING DISCLAIMER. *Codes listed in this policy are for reference purposes only and may not be all- inclusive or applicable for every state or line of business. Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement. Listing of a service or device code in this policy does not guarantee coverage. Coverage is determined by the benefit document. Molina adheres to Current Procedural Terminology (CPT®), a registered trademark of the American Medical Association (AMA). All CPT codes and descriptions are copyrighted by the AMA; this information is included for informational purposes only. Providers and facilities are expected to utilize industry-standard coding practices for all submissions. Molina has the right to reject/deny the claim and recover claim payment(s) if it is determined it is not billed appropriately or not a covered benefit. Molina reserves the right to revise this policy as needed.*

HCPCS CODE	DESCRIPTION
NA	

AVAILABLE DOSAGE FORMS:

Gomekli CAPS 1MG
Gomekli CAPS 2MG
Gomekli TBSO 1MG

REFERENCES

1. Gomekli (mirdametinib) capsules and tablets for oral suspension [prescribing information]. Boston, MA: SpringWorks Therapeutics, Inc.; February 2025.
2. Fisher MJ, et al. Management of neurofibromatosis type 1-associated plexiform neurofibromas.

Neuro Oncol. 2022;24(11):1827–1844. doi:10.1093/neuonc/noac146
3. Moertel CL, et al. ReNeu: a pivotal, Phase IIb trial of mirdametinib in adults and children with symptomatic neurofibromatosis Type 1-associated plexiform neurofibroma [published correction appears in J Clin Oncol. 2025;43(2):239. doi:10.1200/JCO-24-02561.]. J Clin Oncol. doi:10.1200/JCO.24.01034
4. Weiss BD, et al. NF106: a neurofibromatosis clinical trials consortium Phase II trial of the MEK inhibitor mirdametinib (PD-0325901) in adolescents and adults with NF1-related plexiform neurofibromas. J Clin Onc. 2021;39(7)797–806. doi.org/10.1200/JCO.20.02220
5. National Comprehensive Cancer Network. 2025. Central Nervous System Cancers (Version 5.2024). [online] Available at: < cns.pdf> [Accessed 7 April 2025].
6. National Comprehensive Cancer Network. 2026. Central Nervous System Cancers (Version 3.2025). [online] Available at: < cns.pdf> [Accessed 26 December 2025].

SUMMARY OF REVIEW/REVISIONS	DATE
REVISION- Notable revisions: Contraindications/Exclusions/Discontinuation References	Q1 2026
NEW CRITERIA CREATION	Q2 2025