HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Gomekli (mirdametinib)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Gomekli is a kinase inhibitor initially approved by the FDA in 2025. It is indicated for the treatment of neurofibromatosis type 1 (NF1) in patients 2 years or older who have symptomatic plexiform neurofibromatosis not amendable to complete resection. NF1 is a genetic condition that causes various changes to skin pigments and tumor growth on nerve tissue. The tumors can grow anywhere in the nervous system but are typically benign. However, if large tumors are present, it could press onto nerves or organs and cause damage. Gross-total resection is the first-line treatment for NF1 with plexiform neurofibromas (PN). For PN's that cannot be completely removed by surgery, systemic therapy may be appropriate.
Gomekli was approved based on data from the phase IIb ReNeu trial in which adults and children reported significantly PN volume reductions and clinically meaningful improvement in pain.

Gomekli (mirdametinib) will be considered for coverage when the following criteria are met:

Neurofibromatosis Type 1 (NF1)

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with an oncologist or neurologist; AND
3. Member has a diagnosis of neurofibromatosis type 1 (NF1) with at least 1 of the following:
   a) Positive genetic test for NF1
   b) 6 or more café-au-lait macules (CALMs)
   c) Axillary or inguinal freckling
   d) Optic glioma
   e) 2 or more Lisch nodules
   f) A distinctive osseous lesion
   g) Member is the child of a parent diagnosed with NF1; AND
4. Member has a diagnosis of symptomatic plexiform neurofibromatosis; AND
5. Member has at least one measurable plexiform neurofibromas (PN) as evidenced by MRI or PET-CT scan; AND
6. Prescriber attests that the member's disease is not amendable to complete resection; AND
7. Member has received the following evaluation and testing prior to starting therapy:
   a) Comprehensive ophthalmic testing;
   b) Echocardiogram; AND
8. Gomekli is used as monotherapy; AND
9. Member has NOT previously had disease progression with a MEK inhibitor treatment.
10. **Dosage allowed/Quantity limit:** 2 mg/m^2 twice daily for the first 21 days of each 28-day cycle.

QL: 8 mg/day.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must show that the member had at least a partial response (defined as ≥ 20% reduction in PN volume) from baseline and no disease progression and/or
2. Clinical improvement such as reduction of tumor pain or increased physical functioning.

If all the above requirements are met, the medication will be approved for an additional 12 months

HAP CareSource considers Gomekli (mirdametinib) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/05/2025	New policy for Gomekli created.

References:

1. Gomekli (Mirdametinib) [package insert]. Stamford, CT: SpringWorks Therapeutics, Inc.; 2025.
2. Pellerino A, Verdijk RM, Nichelli L, Andratschke NH, Idbaih A, Goldbrunner R. Diagnosis and Treatment of Peripheral and Cranial Nerve Tumors with Expert Recommendations: An EUropean Network for RAre CANcers (EURACAN) Initiative. *Cancers (Basel)*. 2023 Mar 23;15(7):1930. doi: 10.3390/cancers15071930. PMID: 37046591; PMCID: PMC10093509.
3. Neurofibromatosis type 1 – Symptoms and causes. Mayo Clinic. Updated September 10, 2024. Accessed May 5, 2025.
4. Moertel CL, Hirbe AC, Shuhaiber HH, et al. ReNeu: A Pivotal, Phase IIb Trial of Mirdametinib in Adults and Children With Symptomatic Neurofibromatosis Type 1-Associated Plexiform Neurofibroma. J Clin Oncol. 2025 Feb 20;43(6):716-729. doi: 10.1200/JCO.24.01034. Epub 2024 Nov 8. Erratum in: *J Clin Oncol*. 2025 Jan 10;43(2):239. doi: 10.1200/JCO-24-02561. PMID: 39514826; PMCID: PMC11825507.
5. Legius E, Messiaen L, Wolkenstein P, et al. Revised diagnostic criteria for neurofibromatosis type 1 and Legius syndrome: an international consensus recommendation. *Genet Med*. 2021;23(8):1506-1513. doi:10.1038/s41436-021-01170-5

Effective date: 10/01/2025
Revised date: 05/05/2025