



# NCCN Category 2A Recommendation For mirdametinib (GOMEKLI)

**GOMEKLI is the FIRST FDA-approved treatment** for both adults and children 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic plexiform neurofibromas (PN) not amenable to complete resection. GOMEKLI was studied in ReNeu, the first registrational trial in NF1-PN to include both adult and pediatric patients.<sup>1,2</sup>



## What Is NF1-PN?

- Neurofibromatosis type 1 (NF1) is an autosomal dominant genetic disease caused by variants of the *NF1* gene, which encodes neurofibromin, a tumor suppressor protein. The absence of neurofibromin leads to excessive cell growth and tumor formation via MAPK pathway hyperactivation<sup>3</sup>
- Plexiform neurofibromas (PNs) are invasive nerve sheath tumors found in approximately 30%-50% of patients with NF1<sup>4-6</sup>
- Plexiform neurofibromas often cause lifelong morbidities, including pain, disfigurement, impaired physical function, and compression of internal organs, that may be debilitating<sup>4-7</sup>

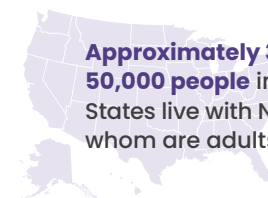
**NCCN**  
CATEGORY 2A

## NATIONAL COMPREHENSIVE CANCER NETWORK® (NCCN®) RECOMMENDATION

NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Central Nervous System Cancers recommend mirdametinib (GOMEKLI) as a Category 2A systemic therapy option for the treatment of adult and pediatric patients ≥2 years of age with NF1 who have symptomatic plexiform neurofibromas not amenable to complete resection.<sup>8</sup>



**NF1-PN is a rare, genetic, progressive, neuro-oncology disease** that impacts both adult and pediatric patients<sup>3-5,9,10</sup>



**Approximately 30,000 to 50,000 people** in the United States live with NF1-PN, most of whom are adults<sup>4-6,11,12</sup>

**GOMEKLI is the FIRST FDA-approved treatment for both adults and children.**

The approval of GOMEKLI provides a meaningful advancement in the treatment of NF1-PN.<sup>1,8</sup>

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MAPK=mitogen-activated protein kinase.

## IMPORTANT SAFETY INFORMATION

### WARNINGS AND PRECAUTIONS

**Ocular Toxicity:** GOMEKLI can cause ocular toxicity including retinal vein occlusion (RVO), retinal pigment epithelium detachment (RPED), and blurred vision. In the adult pooled safety population, ocular toxicity occurred in 28% of patients treated with GOMEKLI: 21% were Grade 1, 5% were Grade 2 and 1.3% were Grade 3. RVO occurred in 2.7%, RPED occurred in 1.3%, and blurred vision occurred in 9% of adult patients. In the pediatric pooled safety population, ocular toxicity occurred in 19% of patients: 17% were Grade 1 and 1.7% were Grade 2. Conduct comprehensive ophthalmic assessments prior to initiating GOMEKLI, at regular intervals during treatment, and to evaluate any new or worsening visual changes such as blurred vision. Continue, withhold, reduce the dose, or permanently discontinue GOMEKLI as clinically indicated.

Please see additional Important Safety Information on next page and [click here](#) for full Prescribing Information.

  
**GOMEKLI®**  
(mirdametinib)  
1 mg tablets for oral suspension  
1 mg and 2 mg capsules

## IMPORTANT SAFETY INFORMATION (CONT'D)

### WARNINGS AND PRECAUTIONS (CONT'D)

**Left Ventricular Dysfunction:** GOMEKLI can cause left ventricular dysfunction. GOMEKLI has not been studied in patients with a history of clinically significant cardiac disease or LVEF <55% prior to initiation of treatment. In the ReNeu study, decreased LVEF of 10 to <20% occurred in 16% of adult patients treated with GOMEKLI. Five patients (9%) required dose interruption, one patient (1.7%) required a dose reduction, and one patient required permanent discontinuation of GOMEKLI. The median time to first onset of decreased LVEF in adult patients was 70 days. Decreased LVEF of 10 to <20% occurred in 25%, and decreased LVEF of  $\geq$ 20% occurred in 1.8% of pediatric patients treated with GOMEKLI. One patient (1.8%) required dose interruption of GOMEKLI. The median time to first onset of decreased LVEF in pediatric patients was 132 days. All patients with decreased LVEF were identified during routine echocardiography, and decreased LVEF resolved in 75% of patients. Before initiating GOMEKLI, assess ejection fraction (EF) by echocardiogram. Monitor EF every 3 months during the first year and then as clinically indicated. Withhold, reduce the dose, or permanently discontinue GOMEKLI based on severity of adverse reaction.

**Dermatologic Adverse Reactions:** GOMEKLI can cause dermatologic adverse reactions including rash. The most frequent rashes included dermatitis acneiform, rash, eczema, maculo-papular rash and pustular rash. In the pooled adult safety population, rash occurred in 92% of patients treated with GOMEKLI (37% were Grade 2 and 8% were Grade 3) and resulted in permanent discontinuation in 11% of patients. In the pooled pediatric safety population, rash occurred in 72% of patients treated with GOMEKLI (22% were Grade 2 and 3.4% were Grade 3) and resulted in permanent discontinuation in 3.4% of patients. Initiate supportive care at first signs of dermatologic adverse reactions. Withhold, reduce the dose, or permanently discontinue GOMEKLI based on severity of adverse reaction.

**Embryo-Fetal Toxicity:** GOMEKLI can cause fetal harm when administered to a pregnant woman. Verify the pregnancy status of females of reproductive potential prior to the initiation of GOMEKLI. Advise pregnant women and females of reproductive potential of the potential risk to a fetus. Also advise patients to use effective contraception during treatment with GOMEKLI and for 6 weeks after the last dose (females) or 3 months after the last dose (males).

### ADVERSE REACTIONS

The most common adverse reactions (>25%) in adult patients were rash (90%), diarrhea (59%), nausea (52%), musculoskeletal pain (41%), vomiting (38%), and fatigue (29%). Serious adverse reactions occurred in 17% of adult patients who received GOMEKLI. The most common Grade 3 or 4 laboratory abnormality (>2%) was increased creatine phosphokinase.

The most common adverse reactions (>25%) in pediatric patients were rash (73%), diarrhea (55%), musculoskeletal pain (41%), abdominal pain (39%), vomiting (39%), headache (34%), paronychia (32%), left ventricular dysfunction (27%), and nausea (27%). Serious adverse reactions occurred in 14% of pediatric patients who received GOMEKLI. The most common Grade 3 or 4 laboratory abnormalities (>2%) were decreased neutrophil count and increased creatine phosphokinase.

### USE IN SPECIFIC POPULATIONS

Verify the pregnancy status of patients of reproductive potential prior to initiating GOMEKLI. Due to the potential for adverse reactions in a breastfed child, advise patients not to breastfeed during treatment with GOMEKLI and for 1 week after the last dose.

### INDICATION

GOMEKLI (mirdametininib) is 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.

**To report SUSPECTED ADVERSE REACTIONS, contact SpringWorks Therapeutics Inc. at 1-888-400-7989 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

Please [click here](#) for full Prescribing Information.

**References:** 1. GOMEKLI. Prescribing Information. SpringWorks Therapeutics, Inc. 2. Moertel CL et al. *J Clin Oncol*. 2025;43(6):716-729. 3. Gutmann DH et al. *Nat Rev Dis Primers*. 2017;3:17004. 4. Prada CE et al. *J Pediatr*. 2012;160(3):461-467. 5. Miller DT et al. *Pediatrics*. 2019;143(5):e20190660. 6. Ejerskov C et al. *Oncol Ther*. 2023;11(1):97-110. 7. Darrigo LG Jr et al. *Brain Behav*. 2022;12(6):e2599. 8. Referenced with permission from the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Central Nervous System Cancers V.2.2025. © National Comprehensive Cancer Network, Inc. 2025. All rights reserved. Accessed October 9, 2025. To view the most recent and complete version of the guideline, go online to [NCCN.org](http://NCCN.org). NCCN makes no warranties of any kind whatsoever regarding their content, use or application and disclaims any responsibility for their application or use in any way. 9. Centers for Medicare & Medicaid Services. Accessed October 9, 2025. <https://www.cms.gov/medicare-coverage-database/view/article.aspx?articleId=59491&ver=7> 10. Centers for Disease Control and Prevention. Accessed October 9, 2025. <https://www.cdc.gov/nchs/icd/icd-10-cm/files.html> 11. Kallionpää RA et al. *Genet Med*. 2018;20(9):1082-1086 12. U.S. Department of Commerce. Accessed October 9, 2025. <https://www.commerce.gov/news/blog/2022/01/us-population-estimated-332403650-jan-1-2022>

