**Sample Letter of Medical Necessity or Medical Exception for GOMEKLI™ (mirdametinib)**

This is an example of a letter to a patient's insurance company supporting the medical necessity or medical exception for GOMEKLI. The information in this letter provides suggestions for the type of information to consider when a letter of medical necessity or medical exception is requested. Use of the information in this letter does not guarantee that the health plan will provide reimbursement, and it is not intended to be a substitute for, or an influence on, the independent medical judgment of the healthcare provider. When completing any request, it is the responsibility of the healthcare provider to adhere to the payor’s specific requirements at that time.

For informational use only.

[Physician letterhead]

Attn: [Insert medical director’s name]

[Insert name of insurance company]

[Insert street address]

[Insert city, state, ZIP]

RE: [Insert patient name]

DOB: [Insert patient’s date of birth]

Policy number: [Insert subscriber policy number]

To whom it may concern:

I am writing on behalf of the above-mentioned patient, [insert patient name], to [document the medical necessity and support coverage for] [request a medical exception to cover] GOMEKLITM (mirdametinib).

The efficacy and safety of GOMEKLI was demonstrated in ReNeu, a multicenter, single-arm, pivotal Phase 2 study (N=114) in patients 2 years of age and older with symptomatic NF1-PN causing significant morbidity. GOMEKLI is the FIRST and ONLY FDA-approved treatment for 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.

[Insert patient name] has been under my care since [date]. Treatment of [insert patient name] with GOMEKLI is medically appropriate and necessary and should be covered and reimbursed based on [insert patient name]’s medical history, diagnosis, and rationale for treatment, as detailed below.

[Clinical considerations for pediatric and adult patients may differ:]

* [Patient’s diagnosis, date of diagnosis, ICD-10-CM diagnosis code(s), condition, and any other relevant medical history]
* [Management/previous therapies used for treating the symptoms associated with plexiform neurofibromas]
* [If patient has difficulty swallowing due to age or tumor growth location and requires liquid suspension]
* [Patient’s response to previous therapies, including reasons for discontinuation]
* [Brief description of the patient’s recent symptoms and conditions (eg, pain, impaired mobility, changes in appearance, organ compression, cognitive deficits, tumor growth)]
* [Summary of your professional opinion explaining the patient’s need for treatment]
* [Additional relevant, medically necessary clinical determinations]

[Consider using this paragraph to include additional clinical information that demonstrates progression of your patient’s plexiform neurofibroma(s), such as documented growth, worsening of symptoms, or impaired functioning in daily life.]

As you consider this request for coverage, please also refer to the enclosed materials for additional information.

[For ease of review, please see below for the location of each enclosure within the submission.]

|  |  |
| --- | --- |
| [History of NF1-PN Diagnosis] | [Document Page Number] |
| * [Document Name]
 | * [X]
 |
| * [Document Name]
 | * [X]
 |
| [Symptoms Management/Previous Therapies Used] | [Document Page Number] |
| * [Document Name]
 | * [X]
 |
| * [Document Name]
 | * [X]
 |
| [Response to Previous Therapies Used] | [Document Page Number] |
| * [Document Name]
 | * [X]
 |
| * [Document Name]
 | * [X]
 |

Please feel free to contact me, [insert physician name], at [insert office phone number], for any additional information you may require. I look forward to receiving your timely response and coverage determination.

Sincerely,

[Insert physician’s name]

* **Suggested Enclosure Documents:**
	+ [GOMEKLI Prescribing Information, published data (such as the pivotal ReNeu study publication)
	+ Clinical practice guidelines or peer-reviewed literature (see the GOMEKLI Evidence Compendium)
	+ Clinical notes/medical records, test results, patient authorization and notice of release of information
	+ Copy of the patient’s health plan or prescription card (front and back)]

**Reference:** GOMEKLI. Prescribing Information. SpringWorks Therapeutics, Inc.

 **INDICATION**

GOMEKLI (mirdametinib) 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.

**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.

 **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 ≥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.

**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.**

**Please** [**click here**](http://springworkstx.com/gomekli-prescribing-info) **for full Prescribing Information including Patient Information and Instructions for Use.**

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