**Sample Letter of Appeal for GOMEKLI**™ **(mirdametinib) to Health Plan Participating in an AFP**

For informational use only.

This is an example of a letter of appeal to a patient's insurance company that participates in an Alternative Funding Program (AFP) and has denied coverage of GOMEKLI. The information in this letter provides suggestions for the type of information to consider including in a letter of appeal. Use of the information in this letter does not guarantee that the health plan will cover GOMEKLI, 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.

[Physician letterhead]

[Date]

Attn: [Insert health insurance plan contact name] RE: [Insert patient name]

[Insert name of insurance company] DOB: [Insert patient date of birth]

[Insert street address] Policy number: [Insert subscriber policy number]

[Insert city, state, ZIP] Group number: [Insert subscriber group number]

[Health plan contact name],

I am writing on behalf of the above-mentioned patient, [insert patient name], to appeal the decision to deny coverage of   
GOMEKLI™ (mirdametinib).

[Patient name] was diagnosed with neurofibromatosis type 1 (NF1) with symptomatic plexiform neurofibromas (PNs) that are not amenable to complete resection on [insert date], and I have prescribed GOMEKLI, a treatment that is medically appropriate and necessary for my patient. GOMEKLI is currently the only treatment approved by the US Food and Drug Administration for [both adults and pediatric patients 2 years of age and older for the treatment of NF1-PN]. Unfortunately, [health plan name] has denied coverage for this medication.

Since denying coverage, it appears that [health plan name] has directed [patient name] to an Alternative Funding Program (AFP) to try and obtain their GOMEKLI medication.

However, after applying through the manufacturer’s Patient Assistance Program (PAP), my patient was notified on [insert date] that they did not meet the program eligibility requirements, and the application for PAP was denied. Additionally, [insert health plan name] is still refusing to cover my patient’s GOMEKLI medication.

Since my patient is not eligible for the PAP and [insert health plan name] has denied coverage, they have no way of accessing their medication and cannot afford to pay out of pocket. Regulations at 45 CFR § 156.122(c) mandate that:

* A health plan providing essential health benefits must have the following processes in place that allow an enrollee, the enrollee’s designee, or the enrollee’s prescribing physician (or other prescriber, as appropriate) to request and gain access to clinically appropriate drugs not otherwise covered by the health plan (i.e., a request for exception);
* In the event that an exception request is granted, the plan must treat the excepted drug(s) as an essential health benefit, including by counting any cost-sharing towards the plan’s annual limitation on cost-sharing under § 156.130 and when calculating the plan’s actuarial value under § 156.135; and
* The health plan must respond within 72 hours and if the medication is deemed medically necessary, the plan is required to cover the prescription for the duration of the plan year, including refills.

In review of the above and the information enclosed, I believe [insert conclusion regarding medical necessity for patient and lack of alternative access to GOMEKLI].

Sincerely,

[Insert physician’s name]

* Enclosures: [clinical documentation, medical literature, patient coverage denial letter, patient PAP denial letter]

**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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