**Sample Letter of Appeal for** **GOMEKLI™ (mirdametinib)**

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| For informational use only. |
| This is an example of information that may be included in an appeal letter to a patient’s insurance company. The information in this letter provides suggestions for the type of information to consider when a letter of appeal is appropriate. 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. |

[Physician letterhead]

[Date]

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]

Group number: [Insert subscriber group number]

Claim number: [Insert patient claim number]

To whom it may concern:

This letter serves as the [select one: 1st /2nd] appeal of the denial for the treatment of my patient, [insert patient name], with GOMEKLITM (mirdametinib). I understand from your denial letter[s] dated [month, day, year] that treatment with GOMEKLI has been denied because [quote denial reason as communicated in the denial letter]. After reviewing the letter[s], I maintain that GOMEKLI is the appropriate treatment for my patient for the reasons detailed below, including [insert patient's name]’s diagnosis and medical history.

[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]

[Some plans may request additional clinical information demonstrating progression of your patient’s plexiform neurofibroma(s). Consider using this paragraph to describe your patient’s worsening of symptoms, impaired functioning in daily life, or other evidence, based on your clinical discretion.]

**Treatment information**

GOMEKLI is the FIRST and ONLY US Food and Drug Administration (FDA)–approved treatment for both 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.1

The primary treatment goals for NF1-PN patients include controlling the size of any plexiform neurofibromas and managing the patient’s symptoms to reduce morbidities.2,3 Achieving these goals most often involves reducing tumor volume or stabilizing tumor growth.2,3 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 with symptomatic NF1-PN causing significant morbidity.1

* [For adult patients, consider mentioning:
  + GOMEKLI is the first and only FDA-approved treatment for adults with NF1-PN. In ReNeu, GOMEKLI delivered confirmed overall response rates by blinded independent central review (41% [n=24/58]; primary endpoint) during the treatment phase, and in the long-term follow-up phase, 2 additional adult patients achieved a confirmed overall response rate, increasing the confirmed overall response rate to 45%1,4
  + Most adult patients treated with GOMEKLI did not experience a dose reduction (83%) or discontinuation (78%) due to an adverse reaction1
  + GOMEKLI is the first and only FDA-approved treatment to deliver deep and durable plexiform neurofibroma tumor volume reduction reaching up to 90% from baseline in adult patients (range, *-*90 to 13) based on a prespecified exploratory analysis4
  + Difficulty swallowing affects about one-fourth of patients with NF1-PN. GOMEKLI has a tablet for oral suspension formulation to help patients with challenges swallowing capsules1,5,6]
* [For pediatric patients, consider mentioning:
  + GOMEKLI is a new FDA-approved treatment option for pediatric patients ≥2 years of age with NF1-PN and delivered confirmed overall response rates by blinded independent central review (52% [n=29/56]; primary endpoint) during the treatment phase, and in the long-term follow-up phase, 1 additional pediatric patient achieved a confirmed overall response rate, increasing the confirmed overall response rate to 54%1,4
  + Most pediatric patients treated with GOMEKLI did not experience a dose reduction (87%) or discontinuation (91%) due to an adverse reaction1
  + GOMEKLI delivered deep and durable plexiform neurofibroma tumor volume reduction from baseline reaching up to 91% in pediatric patients (range, *-*91 to 48) based on a prespecified exploratory analysis2
  + GOMEKLI has a tablet for oral suspension formulation to help pediatric patients with challenges swallowing capsules1,5,6]

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 Name]
* [Document Name]

[Symptoms Management/Previous Therapies Used]

* [Document Name]
* [Document Name]

[Response to Previous Therapies Used]

* [Document Name]
* [Document Name]

[Document Page Number]

* + [X]
  + [X]

[Document Page Number]

* + [X]
  + [X]

[Document Page Number]

* + [X]
  + [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)]

**References: 1.** GOMEKLI. Prescribing Information. SpringWorks Therapeutics, Inc. **2.** Armstrong AE, Belzberg AJ, Crawford JR, Hirbe AC, Wang ZJ. Treatment decisions and the use of MEK inhibitors for children with neurofibromatosis type 1-related plexiform neurofibromas. *BMC Cancer*. 2023;23(1):553. doi:10.1186/s12885-023-10996-y **3.** Fisher MJ, Blakeley JO, Weiss BD, et al. Management of neurofibromatosis type 1-associated plexiform neurofibromas. *Neuro Oncol*. 2022;24(11):1827-1844. doi:10.1093/neuonc/noac146 **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*. Epub ahead of print. doi:10.1200/JCO.24.01034 **5.** Rapado F, Simo R, Small M. Neurofibromatosis type 1 of the head and neck: dilemmas in management. *J Laryngol Otol.* 2001;115(2):151-154. doi:10.1258/0022215011907587 **6.** Yoo HK, Porteous A, Ng A, et al. Impact of neurofibromatosis type 1 with plexiform neurofibromas on the health-related quality of life and work productivity of adult patients and caregivers in the UK: a cross-sectional survey. *BMC Neurol*. 2023;23(1):419. doi:10.1186/s12883-023-03429-7

**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://www.springworkstx.com/gomekli-prescribing-info) **for full Prescribing Information including Patient Information and Instructions for Use.**

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