Treating NF1-PN with GOMEKLI® (mirdametinib):

AUSTIN'S JOURNEY

NF1-PN is a rare, debilitating, progressive, neuro-oncology disease that can impact both adults and children[1-5]

Austin's Challenges With NF1-PN

- Austin's experience with NF1-PN has been characterized by severe pain and limited mobility, leading to frequent visits to the emergency room
- He could not experience activities he enjoyed, such as kayaking, and NF1-PN compromised his career choices
- NF1 runs in Austin's family. Both his dad and grandmother have the disease, but no one in his family had his type of tumors
- Treated with several off-label medications (including pain medicine, sirolimus, sunitinib, and thalidomide)

"On treatment with GOMEKLI, my tumor is smaller."

Patient Case: **Austin**

Age: **30 years old**

Occupation: **Ministry**

GOMEKLI Treatment History: **Taking GOMEKLI since he was 25 years old**

Age at diagnosis: **1 year old**

Austin's Disease and Treatment Journey:

1995 — 2000 — 2020 — 2025

1996

Diagnosed with NF1

1998

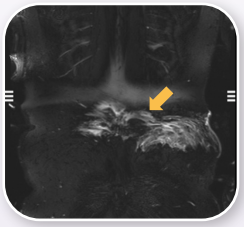
- Dark discoloration on back was seen by his parents and led to a diagnosis of NF1-PN
- Over the years, **multiple plexiform neurofibromas were identified,** including on his left flank

2000

- Underwent **2 debulking surgeries** to remove part of a tumor pressing into his spine

2017

- Pain associated with the plexiform neurofibroma on his left flank increased, along with muscle spasms
- MRI confirmed a large grapefruit-sized plexiform neurofibroma
- **Declined another surgery** due to risks of bleeding, infection, and regrowth given his past experiences



Baseline MRI scan

2020

- Enrolled in the **ReNeu clinical trial** and started taking GOMEKLI
- **Adverse Reactions:** Experienced mild diarrhea, fatigue, and gastritis, mild rash, and Grade 2 palmar-plantar erythrodysesthesia syndrome, which resolved spontaneously within 3 cycles of presentation
- Achieved confirmed overall response. After a year on **GOMEKLI, achieved a 57% reduction in tumor volume**

2024

- Participating in the long-term follow-up phase of the ReNeu trial
- Achieved a **best target tumor volume reduction from baseline of 74%** at Cycle 48
- As a result of tumor volume reduction, Austin's symptoms have improved;* he plans on enjoying more outdoor activities and being engaged in his work

"Those results really motivated me to stay on GOMEKLI. Now, GOMEKLI is just part of my daily routine."

*Based on his most recent scan prior to the June 2024 data cutoff date.

Austin is living with NF1-PN and taking GOMEKLI. This is his story. Every patient's story is different, and individual results vary.

IMPORTANT SAFETY INFORMATION

GOMEKLI is the FIRST FDA-approved treatment for adults and children (≥2 years) with NF1 who have symptomatic PN not amenable to complete resection[6]

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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.

MRI=magnetic resonance imaging; NF1-PN=neurofibromatosis type 1-associated plexiform neurofibromas.

Please see 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

ReNeu Clinical Trial: A pivotal, Phase 2b, single-arm, multicenter trial across 37 US sites[6,7]

The primary end point of the ReNeu trial was confirmed overall response rate during the treatment phase, as assessed by blinded independent central review (BICR).*

- Confirmed overall response rates by BICR were 41% (n=24/58) in adults and 52% (n=29/56) in pediatric patients

*Confirmed overall response rate was defined as the proportion of patients with a complete response (disappearance of the target PN) or partial response (≥20% reduction) on magnetic resonance imaging of the target PN volume from baseline to Cycle 24 (treatment phase) as assessed by blinded independent central review on ≥2 consecutive scans within 2 to 6 months. All responses were partial.[6,7]

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 ≥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 (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.

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 for full Prescribing Information.

CLICK HERE to learn more about patients like Austin and other's experiences with GOMEKLI.

References: 1. Prada CE et al. *J Pediatr*. 2012;160(3):461-467. **2.** Miller DT et al. *Pediatrics*. 2019;143(5):e20190660. **3.** Centers for Medicare & Medicaid Services. Accessed August 1, 2025. https://www.cms.gov/medicare-coverage-database/view/article.aspx?articleId=59125&ver=26 **4.** Centers for Medicare & Medicaid Services. Accessed August 1, 2025. https://www.cms.gov/medicare/coding-billing/icd-10-codes **5.** Gutmann DH et al. *Nat Rev Dis Primers*. 2017;3:17004. **6.** GOMEKLI. Prescribing Information. SpringWorks Therapeutics, Inc. **7.** Moertel CL et al. *J Clin Oncol*. 2025;43(6):716-729.

SpringWorks® THERAPEUTICS

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1 mg and 2 mg capsules