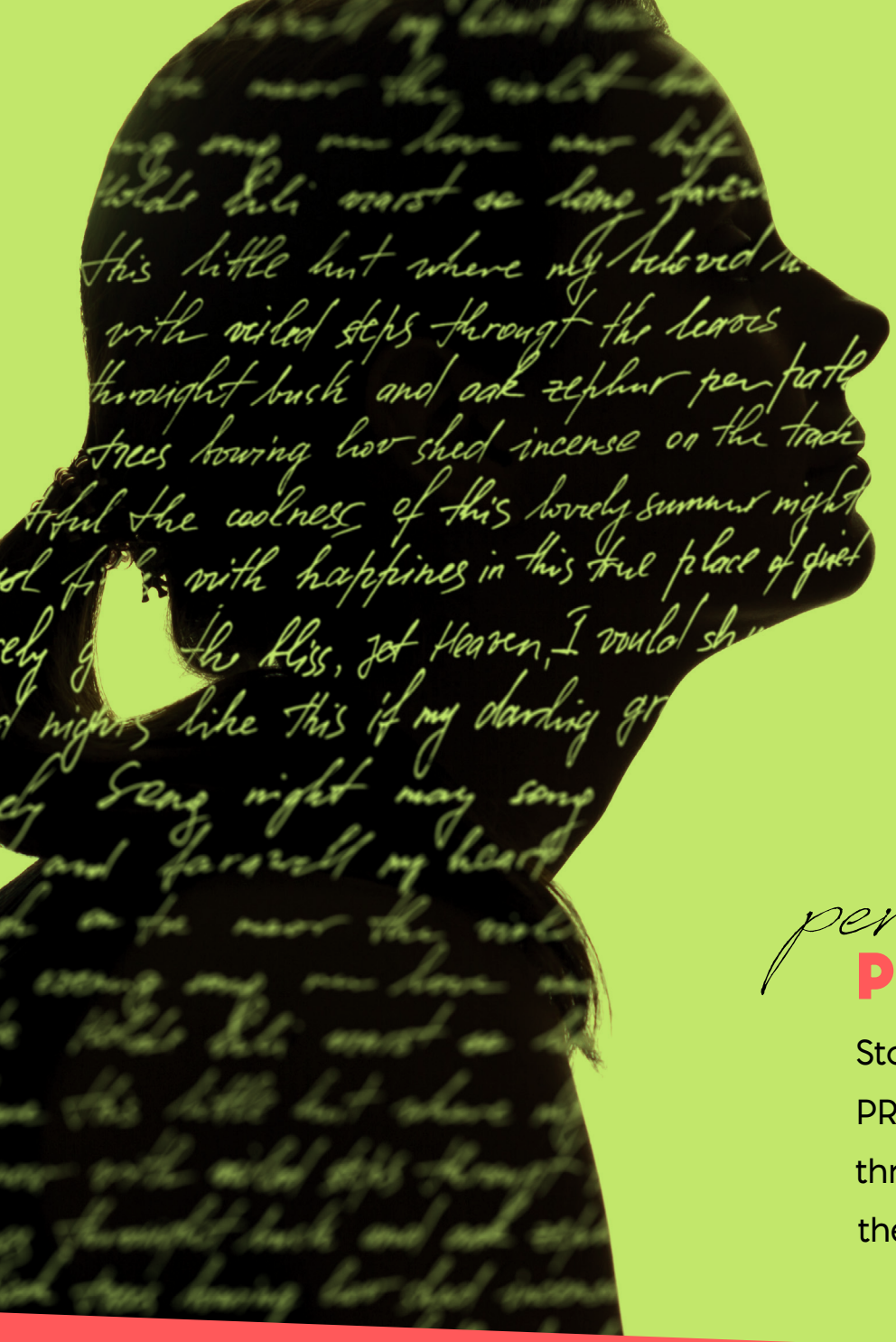


*personal*
**PROSPECTIVES**

Stories of patients with PROS disorders told through a unique lens – their loved ones

## INDICATION

VIJOICE® (alpelisib) tablets is indicated for the treatment of adult and pediatric patients 2 years of age and older with severe manifestations of PIK3CA-Related Overgrowth Spectrum (PROS) who require systemic therapy.

This indication is approved under accelerated approval based on response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

## IMPORTANT SAFETY INFORMATION

VIJOICE is contraindicated in patients with severe hypersensitivity to alpelisib or any of its ingredients.

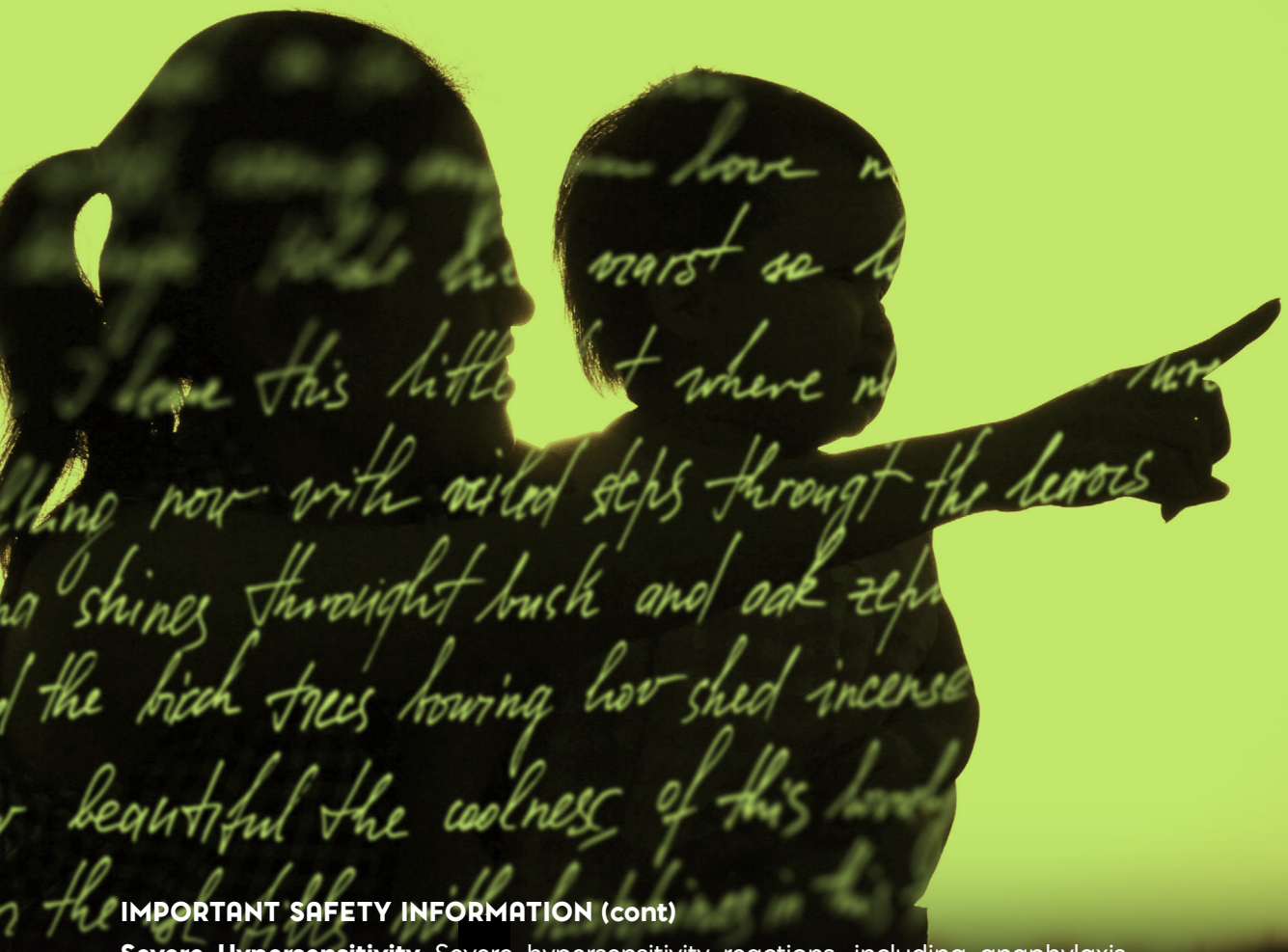
**Please see additional Important Safety Information throughout.**

**Please click here for full Prescribing Information.**

Vijoice®
(alpelisib) tablets
50 mg | 125 mg | 200 mg

## Audrey,
## Ella's mom and caregiver

**"I was very pleased with how quickly we noticed a difference. Within a few weeks, her stomach pain decreased, her mood improved, and she started to engage socially with peers."**



**IMPORTANT SAFETY INFORMATION (cont)**

**Severe Hypersensitivity.** Severe hypersensitivity reactions, including anaphylaxis, angioedema, and anaphylactic shock, have occurred in adult patients treated with alpelisib in the oncology setting and in patients treated with VIJOICE. VIJOICE is not approved for use in the oncology setting. Permanently discontinue VIJOICE in the event of severe hypersensitivity.

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We first began our journey with Klippel-Trenaunay Syndrome (KTS)/PROS when my daughter, Ella, was born. I had a textbook pregnancy with normal ultrasounds and tests. After she was born, the delivery doctor informed us of an "extensive birthmark" on her left side. The doctor told us, "It's probably nothing to worry about, but there's a very small chance it could be a serious vascular condition," and we were referred to the NICU. After 5 stressful days, my daughter was provisionally diagnosed with KTS (mutation later confirmed by tissue analysis) and we finally went home.

I held out hope that Ella's presentation would be mild but as the months progressed, it quickly became clear that her condition was more moderate to severe. Her left abdomen and right buttocks became significantly overgrown to the point where strangers would ask if she had a tumor. At 8 months old, Ella started showing signs of scoliosis, and her weak leg muscles impacted her ability to stand and walk properly. It became clear that we needed to take action, and our vascular anomalies doctors all suggested we begin medical therapy because, in their opinion, the benefits outweighed the risks. I agreed.

At 8 months old, Ella began taking an mTOR inhibitor twice per day, plus an oral antibiotic. I kept waiting to see results but, unfortunately, they never came. Even after 8 months of taking the mTOR inhibitor, there was no improvement in Ella's symptoms. Her overgrowth was still growing aggressively and because she was immunocompromised, she was regularly hospitalized with infections. She became socially withdrawn around other children and would often lie in the fetal position, clutching her stomach as if in pain. One buttocks was so overgrown with lymphatic nodules, she struggled to sit and couldn't bathe in a tub without being held upright.

During this time, we moved to another state and, as a result, switched care teams. Our new vascular anomalies hematologist recommended switching to VIJOICE and took time to explain the benefits and potential risks. While I was nervous about the lack of data for her age group and the potential side effects, I also knew that hyperglycemia can be manageable and I felt reassured by the rigorous testing protocol. Plus, I was eager to help improve my daughter's condition so after consideration, I determined, once again, that the benefits outweighed the risks. Once we petitioned insurance for the necessary approvals, Ella began taking VIJOICE.

I was very pleased with how quickly we noticed a difference. Within a few weeks, her stomach pain decreased, her mood improved, and she started to engage socially with peers. The difference in Ella was noticeable enough that preschool teachers noted the change and asked if we'd done something to her routine or medications. After a couple of months, her overgrowth looked visibly smaller. Her spine started to straighten out. Her shirts and pants became looser. Her right buttocks shrunk, and she could sit in the tub unassisted again. Ella's results have been promising.

My daughter has been on VIJOICE for over a year now and, thankfully, we've not experienced any side effects. Even though the visible rate of improvement has slowed over the past few months, I'm grateful to have medication that has made a difference. Thanks to VIJOICE, my daughter's condition is no longer the first thing people notice about her. They don't comment about the size of her stomach; instead, they mention her happy disposition and how she loves to run and play with friends.

Since KTS/PROS is progressive, I'm not sure what the future will hold. But for now, Ella is thriving. She has part of her childhood back, her laughter, her unfettered spirit, and the hopes that she will be able to participate in future activities. I am forever grateful to Ella's care team for giving us and other PROS families an alternative, nonsurgical treatment that works.

Sincerely, Audrey

# VIJOICE is the FIRST and ONLY FDA-approved treatment for pediatric and adult patients with PROS[1]

*In EPIK-P1, a retrospective study in severe PROS patients who were treated with VIJOICE in a compassionate use program:*

## VIJOICE shrunk overgrowth[1,2]

- 27% of patients (n=10/37) experienced a response at Week 24*† (95% CI, 14-44; response was determined by BICR)
  - 70% of patients maintained response after 6 months and 60% after 12 months

## Safety profile in PROS[1,2]

- The most commonly reported adverse reactions (ARs) were diarrhea (16%), stomatitis (16%), and hyperglycemia (12%)
  - Serious ARs occurred in 12% of patients who received VIJOICE. Dehydration (n=2) and cellulitis (n=2) were the only serious ARs that occurred in multiple patients
    - No patients permanently discontinued treatment due to ARs

*Patients without a response assessment at Week 24 were considered nonresponders. Response was defined as the proportion of patients achieving a ≥20% reduction from baseline in the sum of measurable target lesion volume (1 to 3 lesions) confirmed by at least 1 subsequent imaging assessment, provided that none of the individual target lesions had a ≥20% increase from baseline, nontarget lesions had not progressed, and there were no new lesions.

†Two additional patients experienced a response, but the response was not confirmed by BICR.

BICR, blinded independent central review.

### IMPORTANT SAFETY INFORMATION (cont)

**Severe Cutaneous Adverse Reactions (SCARs).** SCARs, including Stevens-Johnson syndrome (SJS), erythema multiforme (EM), toxic epidermal necrolysis (TEN), and drug reaction with eosinophilia and systemic symptoms (DRESS), have occurred in adult patients treated with alpelisib in the oncology setting and may occur in patients treated with VIJOICE. If signs or symptoms of SCARs occur, interrupt VIJOICE until the etiology of the reaction has been determined. Consultation with a dermatologist is recommended. If a SCAR is confirmed, permanently discontinue VIJOICE. If a SCAR is not confirmed, VIJOICE may require dose modifications, topical or systemic corticosteroids, or oral antihistamine treatment.

**Hyperglycemia.** Severe hyperglycemia, in some cases associated with hyperglycemic hyperosmolar nonketotic syndrome (HHNKS) or fatal cases of ketoacidosis, has occurred in adult patients treated with alpelisib in the oncology setting and may occur in patients treated with VIJOICE.

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**IMPORTANT SAFETY INFORMATION (cont)**

**Hyperglycemia (cont).** In the EPIK-P1 study, grade 1 or 2 hyperglycemia was reported in 12% of patients treated with VIJOICE.

Before initiating treatment with VIJOICE, test fasting plasma glucose (FPG), HbA1c, and optimize blood glucose. After initiating treatment with VIJOICE, monitor fasting glucose (FPG or fasting blood glucose) at least once every week for the first 2 weeks, then at least once every 4 weeks, and as clinically indicated. Monitor HbA1c every 3 months and as clinically indicated. Monitor fasting glucose more frequently for the first few weeks during treatment with VIJOICE in patients with risk factors for hyperglycemia, such as obesity (body mass index ≥30), elevated FPG, HbA1c at the upper limit of normal or above, use of concomitant systemic corticosteroids, or age ≥75.

If a patient experiences hyperglycemia after initiating treatment with VIJOICE, monitor fasting glucose as clinically indicated and at least twice weekly until fasting glucose decreases to normal levels. During treatment with antihyperglycemic medication, continue monitoring fasting glucose at least once a week for 8 weeks, followed by once every 2 weeks, and as clinically indicated. Consider consultation with a health care provider with expertise in the treatment of hyperglycemia, and counsel patients on lifestyle changes.

The safety of VIJOICE in patients with type 1 and uncontrolled type 2 diabetes has not been established. Patients with a history of diabetes mellitus may require intensified hyperglycemic treatment. Closely monitor patients with diabetes.

Interrupt, reduce the dose of, or permanently discontinue VIJOICE based on severity.

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## VIJOICE is the FIRST and ONLY FDA-approved treatment for pediatric and adult patients with PROS[1]

**"Since KTS/PROS is progressive, I'm not sure what the future will hold. But, for now, Ella is thriving."**

-Audrey, Ella's mom and caregiver

**IMPORTANT SAFETY INFORMATION (cont)**

**Pneumonitis.** Severe pneumonitis, including acute interstitial pneumonitis and interstitial lung disease, has occurred in adult patients treated with alpelisib in the oncology setting and may occur in patients treated with VIJOICE.

In patients who have new or worsening respiratory symptoms or are suspected to have developed pneumonitis, interrupt VIJOICE immediately and evaluate the patient for pneumonitis. Consider a diagnosis of noninfectious pneumonitis in patients presenting with nonspecific respiratory signs and symptoms, such as hypoxia, cough, dyspnea, or interstitial infiltrates on radiologic examinations and in whom infectious, neoplastic, and other causes have been excluded by means of appropriate investigations.

Permanently discontinue VIJOICE in all patients with confirmed pneumonitis.

**Diarrhea or Colitis.** Severe diarrhea, resulting in dehydration, and, in some cases, acute kidney injury and colitis, has occurred in adult patients treated with alpelisib in the oncology setting and may occur in patients treated with VIJOICE. In the EPIK-P1 study, 16% of patients experienced grade 1 diarrhea during treatment with VIJOICE. Monitor patients for diarrhea and additional symptoms of colitis, such as abdominal pain and mucus or blood in the stool. Interrupt, reduce the dose of, or permanently discontinue VIJOICE based on the severity of diarrhea or colitis.

**Embryo-Fetal Toxicity.** Based on findings in animals and its mechanism of action, VIJOICE can cause fetal harm when administered to a pregnant woman. Advise pregnant women and females of reproductive potential of the potential risk to a fetus. Advise females of reproductive potential to use effective contraception during treatment with VIJOICE and for 1 week after the last dose. Advise male patients with female partners of reproductive potential to use condoms and effective contraception during treatment with VIJOICE and for 1 week after the last dose.

**The most common adverse reactions (all grades, incidence ≥10%)** were diarrhea (16%), stomatitis (16%), and hyperglycemia (12%).

**The most common laboratory abnormalities (all grades, incidence ≥20%)** were decreased calcium (corrected) (60%), decreased phosphate (59%), increased glucose (56%), increased HbA1c (38%), increased creatinine (31%), increased bilirubin (29%), increased potassium (24%), decreased leukocyte (22%), decreased lymphocyte (20%), and decreased hemoglobin (20%).

The following additional adverse reactions and laboratory abnormality have been identified following administration of VIJOICE: hypersensitivity, lipase increased, dermatitis, and abdominal pain.

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**Please see additional Important Safety Information throughout.**
**Please click here for full Prescribing Information.**

**References: 1.** Vijoice. Prescribing information. Novartis Pharmaceuticals Corp. **2.** Data on file. CBYL719F12002 clinical study report. Novartis Pharmaceuticals Corp; 2021.

NOVARTIS

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