FDA approves Novartis Vijoice® (alpelisib) as first and only treatment for select patients with PIK3CA-Related Overgrowth Spectrum (PROS)

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Vijoice is first approved treatment to specifically address the root cause of PROS conditions in select patients 2 years of age and older[1]

PROS is a spectrum of rare conditions and is characterized by atypical overgrowths and anomalies in blood vessels, the lymphatic system and other tissues[2,3]

Approval based on real-world data from EPIK-P1 study, which showed patients treated with Vijoice experienced reduction in the size of PROS lesions and improvement of PROS-related signs and symptoms

Novartis to offer robust patient support program that includes assistance to access medication, financial resources for eligible patients and continued education

EAST HANOVER, N.J., April 6, 2022 /PRNewswire/ -- Novartis today announced that the U.S. Food and Drug Administration (FDA) granted accelerated approval to Vijoice® (alpelisib) 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.[1] Vijoice is the first FDA-approved treatment for PROS, a spectrum of rare conditions characterized by overgrowths and blood vessel anomalies impacting an estimated 14 people per million.[2,3] In accordance with the Accelerated Approval Program, continued approval may be contingent upon verification and description of clinical benefit from confirmatory evidence.

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"Today's approval of the first treatment for PROS offers hope for a better quality of life to patients and families affected by these rare conditions," said Kristen Davis, Executive Director of CLOVES Syndrome Community. "PROS conditions can be debilitating and disabling and can result in disruptions to everyday activities. Until today, often the only treatment options for patients were surgical or interventional radiology procedures."

PROS conditions can affect quality of life and pose a range of physical, emotional and social challenges for patients and their families, ranging from functional impacts and developmental delays to chronic pain, mobility issues, and feelings of isolation.[3-6] PROS management can be challenging, requiring collaboration from a multidisciplinary team, and patients and physicians have only had access to interventions focused on symptom management.[6,7]

"I am proud of this outstanding achievement for the PROS community. The EPIK-P1 study results build on our earlier pre-clinical findings and demonstrate the efficacy of Vijoice for select PROS conditions, effectively reducing PROS growths," said Guillaume Canaud, MD, PhD, Necker-Enfants Malades Hospital – AP-HP, the Paris Descartes University, Inserm (INEM Institute Necker Enfants Malades – Centre for Molecular Medicine). "This is a significant advancement in therapy for PROS with the potential to positively change the treatment

trajectory and outcomes for patients."

FDA approval was based on real-world evidence from EPIK-P1, a retrospective chart review study that showed patients treated with Vijoice experienced reduced target lesion volume and improvement in PROS-related symptoms and manifestations. The primary endpoint analysis conducted at week 24 showed 27% of patients (10/37) achieved a confirmed response to treatment, defined as 20% or greater reduction in the sum of PROS target lesion volume. Nearly three in four patients with imaging at baseline and week 24 (74%, 23/31) showed some reduction in target lesion volume, with a mean reduction of 13.7%, and no patients experienced disease progression at time of primary analysis. Additionally, at week 24, investigators observed patient improvements in pain (90%, 20/22), fatigue (76%, 32/42), vascular malformation (79%, 30/38), limb asymmetry (69%, 20/29), and disseminated intravascular coagulation (55%, 16/29). These improvements were observed in subsets of patients across the study population (n=57) who reported symptoms at baseline and at week 24.[1,2]

"The approval of Vijoice marks a turning point for patients who, until now, have not had an approved therapy to specifically address their disease," said Victor Bulto, President, Novartis Innovative Medicines US. "We are grateful to the physicians, patients and families who participated in the EPIK-P1 trial. We are continuing to invest in studies to advance the scientific understanding of PROS conditions and to understand the full potential of Vijoice."

In EPIK-P1, the most common adverse events (AEs) of any grade were diarrhea (16%), stomatitis (16%), and hyperglycemia (12%). The most common grade 3/4 AE was cellulitis (4%); one adult case was considered treatment-related.[1]

Novartis is committed to providing patients with access to medicines, as well as resources and support to address a range of needs. The Novartis Oncology Patient Support Program is available to help guide eligible patients through the various aspects of getting started on treatment, from providing educational information to helping them understand their insurance coverage and identify potential financial assistance options. Patients or providers can call 800-282-7630 or visit Patient.NovartisOncology.com or HCP.Novartis.com/Access to learn more about eligibility and to enroll.

About PIK3CA-Related Overgrowth Spectrum (PROS)
The PROS classification was proposed by researchers and parent representatives of patient-family support and advocacy organizations at a National Institutes of Health workshop in 2013 to unite a group of rare overgrowth conditions caused by PIK3CA mutations.[4,6] Specific conditions associated with PROS include KTS, CLOVES syndrome, ILM, MCAP/M–CM, HME, HHML, FIL, FAVA, macrodactyly, muscular HH, FAO, CLAPO syndrome and epidermal nevus, benign lichenoid keratosis, or seborrheic keratosis.[4,6] The estimated prevalence of PROS conditions is approximately 14 people per million.[3]

About Vijoice
Vijoice® (alpelisib) is a kinase inhibitor that treats rare overgrowth conditions caused by the effects of PIK3CA mutations in adults and children with PIK3CA-Related Overgrowth Spectrum (PROS). Vijoice works by inhibiting the PI3K pathway, predominantly the PI3K-alpha isoform.[1] Vijoice is the first FDA-approved treatment for PROS conditions. Vijoice is not approved for use outside the United States.

FDA approval of Vijoice is based primarily on real-world evidence from the EPIK-P1 study. To further understand the long-term efficacy and safety of alpelisib in PROS, Novartis is conducting additional clinical trials. EPIK-P2 is a prospective Phase II multi-center study with a randomized, double-blind, upfront 16-week placebo-controlled period, and extension period to evaluate the safety, the efficacy and pharmacokinetics of alpelisib to treat pediatrics and adults with PROS. EPIK-P3 is a Phase II study to assess long-term safety and

efficacy of alpelisib in people with PROS who participated in EPIK-P1.

Important Safety Information

Indication
VIJOICE® (alpelisib) tablets is a prescription medicine used to treat 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
Patients should not take VIJOICE if they have had a severe allergic reaction to alpelisib or are allergic to any of the ingredients in VIJOICE.

VIJOICE may cause serious side effects. VIJOICE can cause severe allergic reactions. Patients should tell their health care provider or get medical help right away if they have trouble breathing, flushing, rash, fever, or fast heart rate during treatment with VIJOICE. VIJOICE can cause severe skin reactions. Patients should tell their health care provider or get medical help right away if they get a severe rash or a rash that keeps getting worse; reddened skin; flu-like symptoms; blistering of the lips, eyes, or mouth; blisters on the skin or peeling skin, with or without a fever. VIJOICE can cause high blood sugar levels (hyperglycemia). Hyperglycemia is common with VIJOICE and can be severe. Health care providers will monitor blood sugar levels before patients start, and during treatment with, VIJOICE. Health care providers may monitor blood sugar levels more often if patients have a history of type 2 diabetes. Patients should tell their health care provider right away if they develop symptoms of hyperglycemia or its complications, including excessive thirst, dry mouth, urinating more often than usual or having a higher amount of urine than normal, increased appetite with weight loss, confusion, nausea, vomiting, fruity odor on breath, difficulty breathing, or dry or flushed skin. VIJOICE can cause lung problems (pneumonitis). Patients should tell their health care provider right away if they develop new or worsening symptoms of lung problems, including shortness of breath or trouble breathing, cough, or chest pain. Diarrhea is common with VIJOICE and can be severe. Severe diarrhea can lead to the loss of too much body water (dehydration) and kidney injury. Patients who develop diarrhea during treatment with VIJOICE should tell their health care provider right away.

Before taking VIJOICE, patients should tell their health care provider if they have a history of diabetes; skin rash; redness of skin; blistering of the lips, eyes, or mouth; peeling skin; are pregnant, or plan to become pregnant, because VIJOICE can harm their unborn baby. Females who can become pregnant should use effective birth control during treatment with VIJOICE and for 1 week after the last dose. Do not breastfeed during treatment with VIJOICE and for 1 week after the last dose. Males with female partners who can become pregnant should use condoms and effective birth control during treatment with VIJOICE and for 1 week after the last dose.

Patients should tell their health care provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. VIJOICE and other medicines may affect each other and cause side effects. Know the medicines you take. Keep a list of them to show your health care provider or pharmacist when you get a new medicine.

The most common side effects of VIJOICE are diarrhea, mouth sores (stomatitis), and hyperglycemia.

Please see full Prescribing Information for VIJOICE, available at VIJOICE.com.

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List of links present in page

1. https://prod1.novartis.com/us-en/us-en/news/media-releases/fda-approves-novartis-vijoice-alpelisib-first-

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