Vijoice (alpelisib)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Vijoice (alpelisib) drug policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Vijoice 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.

POLICY

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

1. For initial requests: Documentation of test confirming presence of PIK3CA mutation
2. For continuation requests: Chart notes or medical record documentation supporting positive clinical response.

Criteria for Initial Approval

PIK3CA-Related Overgrowth Spectrum (PROS)

Authorization of 6 months may be granted for treatment of PROS when all of the following criteria are met:

1. The member is at least 2 years of age

2. The member has severe manifestations of disease and requires systemic therapy
3. The member has a PIK3CA mutation

Continuation of Therapy
Authorization of 12 months may be granted for all members (including new members) requesting continuation of therapy with Vijoice when all initial criteria is met and there is no evidence of unacceptable toxicity or disease progression while on the current regimen.

Vijoice (alpelisib) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Dosing and Administration
Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits
Vijoice – 60 tablets per 30 days

CLINICAL RATIONALE

Vijoice (alpelisib) is indicated for the treatment of adult and pediatric patients 2 years of age and older with severe manifestations of phosphatidylinositol-4,5-bisphosphate 3-kinase α (*PIK3CA*)-related overgrowth spectrum (PROS) who require systemic therapy. PROS includes a rare group of genetic disorders that leads to overgrowth of various body parts due to activating mutations in the *PIK3CA* gene. The overgrowth usually occurs asymmetrically, and the predominantly affected areas include the brain, limbs (including fingers and toes), trunk, and face. Different tissues (e.g., fat, muscle, bone, nerve, brain, and blood vessels) may be involved individually or in combination.

Treatment for PROS is primarily based on supportive care, including surgery, sclerotherapy, and psychological and nutritional support. Since PIK3CA recruits the amphiregulin (AKT)/mechanistic target of rapamycin (mTOR) pathway, some patients are treated with mTOR inhibitors (e.g., sirolimus), which are associated with some level of improvement in vascular malformations; however, these agents have moderate and inconsistent effects on the volume of the overgrowth and are associated with significant adverse events. The 2021 international expert consensus statement for PIK3CA-related disorders states a PIK3CA inhibitor may be considered for PROS with severe/intractable endocrinopathies (evidence score C: no evidence or consensus agreement/not currently specified as best practice).

An unpublished, single-arm, retrospective, medical chart review study in patients with PROS who were treated with Vijoice (alpelisib) as compassionate use under a managed access program was conducted at seven sites in five countries (i.e., Australia, France, Ireland, Spain, United States) (EPIK-P1; N = 57 [39 pediatric patients (median age: 10 years, range: 2 years to 17 years); 18 adult patients (median age: 26 years, range: 18 years to 50 years)]; median 14 years from diagnosis; 57% were female; 11% were White and race was not reported for 89%; N = 37 for efficacy population). Other baseline characteristics included that 93% had congenital overgrowth and 98% had heterogenous manifestations, including CLOVES (81%), MCAP (8%), KTS (3%), FIL (8%), and other (5%). Patients 2 years of age and older with PROS with a confirmed *PIK3CA* mutation and severe and/or life-threatening condition requiring treatment who received at least one dose of Vijoice (alpelisib) for compassionate use were included in the study. Adults received Vijoice (alpelisib) 250 mg orally per day, and pediatric patients received Vijoice (alpelisib) 50 mg orally per day for at least 24 weeks. The primary endpoint was the proportion of patients with radiological response (i.e., patients with ≥ 20% reduction in the sum of target lesion volume from the start of Vijoice [alpelisib] therapy) at week 24. The median length Vijoice (alpelisib) therapy was 18.1 months. Vijoice (alpelisib) therapy resulted in a 27% response rate at week 24 with 60% of patients with a duration of response for at

least 12 months. Of the 31 patients with imaging at index and week 24, 23 patients (74%) experienced reduction in sum of target lesion volume with a mean reduction of 14%. In the first 24 weeks, there were no surgeries due disease progression. The safety profile was favorable compared with Piqray (alpelisib) in the oncology setting; adverse events were mild, transient, and manageable with appropriate intervention. Overall, treatment with Vijoice (alpelisib) resulted in a 27% response rate, reduction in lesion volume, and improvement in PROS-related surgeries.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- N/A

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POLICY HISTORY

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