MEDICAL MUTUAL®

Drug Policy

Policy:	Vijoice (alpelisib)	Annual Review Date: 05/16/2024 Last Revised Date: 05/16/2024

OVERVIEW

Vijoice, a kinase inhibitor, is indicated for the treatment of adults and pediatric patients ≥ 2 years of age with severe manifestations of phosphatidylinositol- 4,5-bisphosphate 3-kinase catalytic subunit alpha **(PIK3CA)-Related Overgrowth Spectrum** (PROS) who require systemic therapy.

POLICY STATEMENT

This policy involves the use of Vijoice. Prior authorization is recommended for pharmacy benefit coverage of Vijoice. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Vijoice as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Vijoice be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Vijoice is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **PIK3CA-Related Overgrowth Spectrum (PROS).** Approve for the duration noted if the patient meets ONE of the following criteria (A or B):
   Note: Examples of PROS include congenital lipomatous overgrowth, vascular malformations, epidermal nevi, scoliosis/skeletal and spinal (CLOVES) syndrome; megalencephaly-capillary malformation (MCAP) syndrome; Klippel-Trenaunay syndrome (KTS); facial infiltrating lipomatosis (FIL), dysplastic megalencephaly (DMEG); hemimegalencephaly (HMEG); focal cortical dysplasia (FCD); or capillary vascular malformation of the lower lip, lymphatic malformations of the head and neck, asymmetry and partial or generalized overgrowth (CLAPO) syndrome.
   A) Initial Therapy. Approve for 6 months if the patient meets the following criteria (i, ii, iii, and iv):
      i. Patient is ≥ 2 years of age; AND

ii. Patient has at least one severe clinical manifestation of PROS, as determined by the prescriber; AND
Note: Examples of severe clinical manifestations include excessive tissue growth, blood vessel malformations, scoliosis, vascular tumors, cardiac or renal manifestations, and those that require systemic treatment.

iii. Patient has a *PIK3CA* mutation as confirmed by genetic testing; AND

iv. The medication is being prescribed by or in consultation with a physician that specializes in treatment of genetic disorders.

B) Patient is Currently Receiving Vijoice. Approve for 1 year if the patient meets the following criteria (i, ii and iii):

i. Patient has been established on Vijoice for at least 6 months; AND
Note: A patient who has received < 6 months of therapy or who is restarting therapy with Vijoice is reviewed under criterion A (Initial Therapy).

ii. Patient has experienced a reduction in volume from baseline (prior to initiating Vijoice) in at least one lesion, as confirmed by measurement; AND

iii. Patient has experienced an improvement in at least one sign or symptom of PROS from baseline (prior to initiating Vijoice).
Note: Examples of signs or symptoms of PROS include pain, fatigue, vascular malformation, limb asymmetry, or disseminated intravascular coagulation.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 180 days
B) *Extended Approval:* 365 days

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Vijoice has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:
The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

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3. Canaud G, Lopez Gutierrez JC, Irvine A, et al. EPIK-P1: retrospective chart review study of patients with PIK3CA-related overgrowth spectrum who have received alpelisib as part of a compassionate use programme [abstract]. Presented at : The 2021 European Society for Medical Oncology (ESMO) Congress; virtual; September 16-21, 2021.
4. Keppler-Noreuil K, Rios JJ, Parker V, et al. PIK3CA-related overgrowth spectrum (PROS): diagnostic and testing eligibility criteria, differential diagnosis, and evaluation. *Am J Med Genet A*. 2015;0(2):287-295.
5. National Center of Advancing Translational Sciences. Genetic and Rare Disease Information Center. PIK3CA-related overgrowth spectrum. Available at: https://rarediseases.info.nih.gov/diseases/12182/pik3ca-related-overgrowth-spectrum. Created January 29, 2018. Accessed on April 11, 2022.
6. Canuad G, Hammil AM, Adams D. A review of mechanisms of disease across PIK3CA-related disorders with vascular manifestations. *Orphanet J Rare Dis*. 2021 July 8. [Epub ahead of print].