HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Vijoice (alpelisib)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Vijoice, approved by the FDA in 2022, is a phosphatidylinositol-3-kinase (PI3K) inhibitor 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. It was approved under the FDA accelerated approval pathway and is the first approved treatment for PROS. Existing treatment strategies include surgery, interventional radiology, and symptom management.
PROS is a group of rare conditions characterized by overgrowth of various parts of the body, caused by mutations in the *PIK3CA* gene which has a role in regulating cell growth and division. Mutation can result in uncontrolled growth of the affected tissue(s). Some examples of PROS are Congenital Lipomatous Overgrowth, Vascular Malformations, Epidermal Nevi, Scoliosis/Skeletal and Spinal syndrome (CLOVES), Megalencephaly-Capillary Malformation Polymicrogyria (MCAP), Klippel-Trenaunay Syndrome (KTS), Facial Infiltrating Lipomatosis (FIL).
Vijoice was studied in the EPIK-P1 clinical trial, a retrospective chart review in 37 patients. In the study, 27% had a radiological response at week 24, and 60% had a response lasting at least 12 months.
Of note, alpelisib is marketed under the brand name Piqray for the treatment of breast cancer.

Vijoice (alpelisib) will be considered for coverage when the following criteria are met:

PIK3CA-Related Overgrowth Spectrum (PROS)

For **initial** authorization:
1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with an oncologist, geneticist, metabolic specialist, or dermatologist; AND
3. Member has a diagnosis of a PROS disorder confirmed by documentation of both of the following:
   a) Mutation of the *PIK3CA* gene*
   b) At least one measurable target lesion identified on imaging; AND
4. The physician has determined the member's condition is severe, life-threatening, or requires surgical intervention.
5. **Dosage allowed/Quantity limit:**
   Adults (18+): 250 mg by mouth once daily
   Age 2 to <6 years: 50 mg once daily
   Age 6 to <18 years: 50 mg once daily; may consider increase to 125 mg once daily after 24 weeks.
   QL: 56 tablets per 28 days OR 28 packets of granules per 28 days

If all the above requirements are met, the medication will be approved for 6 months.

Note: Although not all patients may present with a mutation, the drug was only studied in patients who met this criterion. There is no data available for using Vijoice in those without the mutation.

For **reauthorization**:

1. Documentation must be provided to show at least 20% reduction in the sum of measurable target lesion volume from baseline; AND
2. No progression of non-target lesions; AND
3. No appearance of new lesions.

If all the above requirements are met, the medication will be approved for an additional 6 months.

HAP CareSource considers Vijoice (alpelisib) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
06/21/2022	New policy for Vijoice created.
09/05/2024	Updated references. Added granules with QL as a new formulation.

References:

1. Vijoice. Prescribing information. Novartis Pharmaceuticals Corporation; 2024.
2. Keppler-Noreuil KM, Rios JJ, Parker VE, et al. PIK3CA-related overgrowth spectrum (PROS): diagnostic and testing eligibility criteria, differential diagnosis, and evaluation. *Am J Med Genet A*. 2015;167A(2):287-295. doi:10.1002/ajmg.a.36836
3. Canaud G, Hammill AM, Adams D, Vikkula M, Keppler-Noreuil KM. A review of mechanisms of disease across PIK3CA-related disorders with vascular manifestations. *Orphanet J Rare Dis*. 2021;16(1):306. Published 2021 Jul 8. doi:10.1186/s13023-021-01929-8
4. Venot Q, Blanc T, Rabia SH, et al. Targeted therapy in patients with PIK3CA-related overgrowth syndrome [published correction appears in Nature. 2019 Apr;568(7752):E6]. *Nature*. 2018;558(7711):540-546. doi:10.1038/s41586-018-0217-9
5. Douzgou S, Rawson M, Baselga E, et al. A standard of care for individuals with PIK3CA-related disorders: An international expert consensus statement. *Clin Genet*. 2022;101(1):32-47. doi:10.1111/cge.14027
6. Mirzaa G, Graham JM Jr, Keppler-Noreuil K. PIK3CA-Related Overgrowth Spectrum. 2013 Aug 15 [Updated 2023 Apr 6]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: https://www.ncbi.nlm.nih.gov/books/NBK153722/
7. Gazzin A, Leoni C, Viscogliosi G, et al. Work-Up and Treatment Strategies for Individuals with PIK3CA-Related Disorders: A Consensus of Experts from the Scientific Committee of the Italian Macrodactyly and PROS Association. *Genes (Basel)*. 2023;14(12):2134. Published 2023 Nov 27. doi:10.3390/genes14122134

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