Vijoice® (alpelisib) – New orphan drug approval

- On April 6, 2022, Novartis announced the FDA approval of 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.
    - 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 a confirmatory trial(s).
- PROS are a spectrum of rare conditions characterized by overgrowths and blood vessel anomalies. The estimated prevalence of PROS conditions is approximately 14 people per million.
- Alpelisib is also approved under the brand name Piqray® for use in combination with fulvestrant for the treatment of postmenopausal women, and men, with hormone receptor-positive, human epidermal growth factor receptor 2 (HER2)-negative, PIK3CA-mutated, advanced or metastatic breast cancer.
- The efficacy of Vijoice was established in EPIK-P1, a single-arm clinical study in 37 patients who were treated as part of an expanded access program for compassionate use which enrolled patients across seven sites in five countries (France, Spain, U.S., Ireland and Australia). The major efficacy outcome measure was the proportion of patients with radiological response at week 24, defined as a ≥ 20% reduction from baseline in the sum of measurable target lesion volume (1 to 3 lesions) confirmed by at least one subsequent imaging assessment, in the absence of a ≥ 20% increase from baseline in any target lesion, progression of non-target lesions, or appearance of a new lesion.
    - The response rate at week 24 was 27% (95% CI: 14, 44).
- Warnings and precautions for Vijoice include severe hypersensitivity, severe cutaneous adverse reactions, hyperglycemia, pneumonitis, diarrhea, and embryo-fetal toxicity.
- The most common adverse reactions (grades 1 to 4, incidence ≥ 10%) with Vijoice use were diarrhea, stomatitis, and hyperglycemia.
- The recommended dosage of Vijoice in adult patients is 250 mg orally, once daily, administered as recommended until disease progression or unacceptable toxicity.
- The recommended initial dosage of Vijoice in pediatric patients is 50 mg orally, once daily, administered as recommended until disease progression or unacceptable toxicity.
    - A dose increase to 125 mg once daily can be considered in pediatric patients ≥ 6 years old for response optimization (clinical/radiological) after 24 weeks of treatment with Vijoice at 50 mg once daily. When a pediatric patient turns 18 years old, a gradual dose increase up to 250 mg can be considered.
- Novartis' launch plans for Vijoice are pending. Vijoice will be available as 50 mg, 125 mg, and 200 mg tablets.