Voranigo® (vorasidenib) – New orphan drug approval

- On August 6, 2024, Servier Pharmaceuticals announced the FDA approval of Voranigo (vorasidenib), for the treatment of adult and pediatric patients 12 years and older with grade 2 astrocytoma or oligodendroglioma with a susceptible isocitrate dehydrogenase-1 (IDH1) or isocitrate dehydrogenase-2 (IDH2) mutation following surgery including biopsy, sub-total resection, or gross total resection.

- Gliomas are types of brain cancer that can hinder normal brain function and cause a variety of symptoms.

- Voranigo is an IDH1/IDH2 inhibitor.

- The efficacy of Voranigo was established in INDIGO, a randomized, double-blind, placebo-controlled study in 331 patients with IDH1- or IDH2-mutant grade 2 astrocytoma or oligodendroglioma with prior surgery including biopsy, sub-total resection, or gross total resection. Patients were randomized to Voranigo or placebo. The major efficacy outcome was progression-free survival (PFS).

    — Median PFS was 27.7 months in the Voranigo group vs. 11.1 months in the placebo group (hazard ratio, 0.39; 95% CI: 0.27, 0.56; $p < 0.001$).

- Warnings and precautions for Voranigo include hepatotoxicity and embryo-fetal toxicity.

- The most common adverse reactions (≥ 15%) with Voranigo use were fatigue, headache, COVID-19, musculoskeletal pain, diarrhea, nausea, and seizure. The most common (≥ 2%) grade 3 or 4 laboratory abnormalities were increased alanine aminotransferase, increased aspartate aminotransferase, increased gamma-glutamyl transferase, and decreased neutrophils.

- The recommended dosage of Voranigo in adult patients is 40 mg orally once daily until disease progression or unacceptable toxicity.

- The recommended dosage of Voranigo in pediatric patients 12 years and older is based on body weight:

    — Patients weighing ≥ 40 kg: 40 mg orally once daily
    — Patients weighing <40 kg: 20 mg orally once daily
    — Treatment should be continued until disease progression or unacceptable toxicity.

- Patients should be selected for Voranigo treatment based on the presence of IDH1 or IDH2 mutations in tumor specimens.

    — An FDA-approved test for detection of IDH1 or IDH2 mutations in grade 2 astrocytoma or oligodendroglioma for selecting patients for treatment with Voranigo is not available.

- Servier Pharmaceuticals' launch plans for Voranigo are pending. Voranigo will be available as 10 mg and 40 mg tablets.