Zelboraf® (vemurafenib) – New indication

- On November 6, 2017, the FDA announced the approval of Genentech's Zelboraf (vemurafenib) for the treatment of patients with Erdheim-Chester disease (ECD) with BRAF V600 mutation.
    - Zelboraf is also approved for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E mutation as detected by an FDA-approved test.
    - Zelboraf is not indicated for treatment of patients with wild-type BRAF melanoma.
- ECD is a slow-growing blood cancer that originates in the bone marrow. ECD causes an increased production of histiocytes, a type of white blood cell, which can result in tumors infiltrating organs and tissues.
    - ECD is estimated to affect 600 – 700 patients worldwide. Approximately 54% of patients with ECD have the BRAF V600 mutation.
- The new indication for Zelboraf was approved based on a single-arm trial in 22 patients (≥ 16 years old) with BRAF V600 mutation-positive ECD. The primary endpoint was the objective response rate (ORR).
    - In the trial, the ORR was 54.5% (95% CI: 32.2, 75.6). In addition, 50% of the patients achieved a partial response and 4.5% of patients achieved a complete response.
- In the ECD trial, the most common adverse reactions (> 50%) with Zelboraf use were arthralgia, maculo-papular rash, alopecia, fatigue, prolonged QT interval, and skin papilloma.
- In patients with ECD, the recommended dosage of Zelboraf is 960 mg (four 240 mg tablets) orally every 12 hours until disease progression or toxicity.
    - For the dosage of Zelboraf in melanoma, please refer to the drug label.