Retevmo® (selpercatinib) – New indication

- On September 21, 2022, Eli Lilly announced the FDA approval of Retevmo (selpercatinib), for the treatment of adult patients with locally advanced or metastatic solid tumors with a *rearranged during transfection (RET)* gene fusion that have progressed on or following prior systemic treatment or who have no satisfactory alternative treatment options.
    - This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).
- In addition to the tumor-agnostic approval, the FDA has granted traditional approval for Retevmo in adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) with a *RET* gene fusion, as detected by an FDA-approved test.
    - This FDA action broadens the Retevmo label to include patients with locally advanced disease and converts the May 2020 accelerated approval for NSCLC to a traditional approval.
- Retevmo is also approved via accelerated approval for *RET*-mutant medullary thyroid cancer and *RET* fusion-positive thyroid cancer.
- The approval of Retevmo for the new indication was based on an open-label, multi-cohort trial (LIBRETTO-001) in patients with locally advanced or metastatic *RET* fusion-positive solid tumors. Efficacy was evaluated in 41 patients with *RET* fusion-positive tumors other than NSCLC and thyroid cancer with disease progression on or following prior systemic treatment or who had no satisfactory alternative treatment options. The outcome measures were overall response rate (ORR) and duration of response (DOR).
    - The ORR was 44% (95% CI: 28, 60).
    - The median DOR was 24.5 months (95% CI: 9.2, not estimable).
- The updated drug label also includes additional data from LIBRETTO-001 in patients with advanced or metastatic *RET* fusion-positive NSCLC. Efficacy was evaluated in 247 patients with *RET* fusion-positive NSCLC previously treated with platinum chemotherapy and 69 patients with treatment-naïve *RET* fusion-positive NSCLC.
    - In previously treated patients, the ORR was 61% (95% CI: 55, 67) with a median DOR of 28.6 months (95% CI: 20, not estimable).
    - In treatment-naïve patients, the ORR was 84% (95% CI: 73, 92) with a median DOR of 20.2 months (95% CI: 13, not estimable).
- The recommended dose of Retevmo for all its uses is 120 mg or 160 mg (for less than 50 kg body weight or 50 kg or greater, respectively) orally twice daily until disease progression or unacceptable toxicity