Tepezza™ (teprotumumab-trbw) – New orphan drug approval

- On January 21, 2020, the FDA announced the approval of Horizon Therapeutics' Tepezza (teprotumumab-trbw), for the treatment of thyroid eye disease (TED).

- TED is a progressive and vision-threatening rare autoimmune disease. TED is associated with the outward bulging of the eye (proptosis) that can cause a variety of symptoms such as eye pain, double vision, light sensitivity or difficulty closing the eye.

- Tepezza is an insulin-like growth factor-1 receptor inhibitor (IGF-1R). The mechanism of action in patients with TED has not been fully characterized.

- Tepezza was evaluated in two randomized, double-masked, placebo-controlled studies in 171 patients with TED. Patients were given intravenous (IV) infusions every 3 weeks for a total of 8 infusions. The proptosis responder rate at week 24 was defined as the percentage of patients with ≥ 2 mm reduction in proptosis in the study eye from baseline, without deterioration in the non-study eye (≥ 2 mm increase) in proptosis.

    - In study 1, the proptosis responder rate was 71% and 20% with Tepezza and placebo, respectively (difference: 51, 95% CI: 33, 69).
    - In study 2, the proptosis responder rate was 83% and 10% with Tepezza and placebo, respectively (difference: 73, 95% CI: 59, 88).

- Warnings and precautions for Tepezza include infusion reactions, exacerbation of inflammatory bowel disease, and hyperglycemia.

- The most common adverse reactions (> 5%) with Tepezza use were muscle spasm, nausea, alopecia, diarrhea, fatigue, hyperglycemia, hearing impairment, dry skin, dysgeusia and headache.

- The recommended dose of Tepezza is 10 mg/kg for the first IV infusion, followed by 20 mg/kg every 3 weeks for 7 additional infusions.

- Horizon Therapeutics plans to launch Tepezza in the coming weeks. Tepezza will be available as a 500 mg lyophilized powder in a single-dose vial for reconstitution