Evkeeza™ (evinacumab) – New orphan drug approval

- On February 11, 2021, Regeneron announced the FDA approval of Evkeeza (evinacumab), as an adjunct to other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adult and pediatric patients, aged 12 years and older, with homozygous familial hypercholesterolemia (HoFH).
    - The safety and effectiveness of Evkeeza have not been established in patients with other causes of hypercholesterolemia, including those with heterozygous familial hypercholesterolemia.
    - The effects of Evkeeza on cardiovascular morbidity and mortality have not been determined.
- HoFH is an ultra-rare inherited condition that affects approximately 1,300 patients in the U.S. HoFH occurs when two copies of the FH-causing genes are inherited, one from each parent, resulting in high levels of LDL-C. Patients with HoFH are at risk for premature atherosclerotic disease and cardiac events as early as their teenage years.
- Evkeeza is a first-in-class monoclonal antibody that binds to and blocks the function of angiopoietin-like protein 3 (ANGPTL3). ANGPTL3 is a member of the angiopoietin-like protein family that is expressed primarily in the liver and plays a role in the regulation of lipid metabolism.
- The efficacy of Evkeeza was established in ELIPSE-HoFH, a double-blind, randomized, placebo-controlled study in 65 patients with HoFH. Patients were on a background of other lipid-lowering therapies, including maximally tolerated statins, ezetimibe, PCSK9 inhibitor antibodies, Juxtapid® (lomitapide), and lipoprotein apheresis. The primary efficacy endpoint was percent change in LDL-C from baseline to week 24.
    - At week 24, the least squares mean treatment difference between Evkeeza and placebo in mean percent change in LDL-C from baseline was -49% (95% CI: -65, -33% $p < 0.0001$).
- Warnings and precautions for Evkeeza include serious hypersensitivity reactions and embryo-fetal toxicity.
- The most common adverse reactions (≥ 5%) with Evkeeza use were nasopharyngitis, influenza-like illness, dizziness, rhinorrhea, and nausea.
- The recommended dose of Evkeeza is 15 mg/kg administered by intravenous (IV) infusion over 60 minutes once monthly (every 4 weeks).
    - LDL-C should be assessed when clinically appropriate. The LDL-lowering effect of Evkeeza may be measured as early as 2 weeks after initiation.
- The average Wholesale Acquisition Cost (WAC) per patient for Evkeeza will vary based on weight, and is expected to be approximately $450,000 per year on average.

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- Regeneron’s launch plans for Evkeeza are pending. Evkeeza will be available as 345 mg/2.3 mL (150 mg/mL) and 1,200 mg/8 mL (150 mg/mL) solutions in single-dose vials.

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