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FDA approves TEPEZZATM to treat Thyroid Eye Disease

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First and only FDA-approved medicine for TED, a serious, progressive, vision-threatening rare disease

Ireland headquartered Horizon Therapeutics plc has announced that the U.S. Food and Drug Administration (FDA) has approved TEPEZZA^{TM} (teprotumumab-trbw) for the treatment of Thyroid Eye Disease (TED). TEPEZZA is the first and only FDA-approved medicine for the treatment of TED, a serious, progressive and vision-threatening rare autoimmune disease that is associated with proptosis (eye bulging), diplopia (double vision), blurred vision, pain, inflammation and facial disfigurement. TEPEZZA is a fully human monoclonal antibody (mAb) and a targeted inhibitor of the insulin-like growth factor-1 receptor (IGF-1R) that is administered to patients once every three weeks for a total of eight infusions.

“Today is a great day for people living with Thyroid Eye Disease, a rare, vision-threatening disease that previously had no FDA-approved treatment options,” said Timothy Walbert, chairman, president and chief executive officer, Horizon. “The TED community has gone far too long without an FDA-approved therapy, and we are grateful to the people living with TED and physicians who partnered with us on the clinical development program that led to today’s approval of TEPEZZA. This also marks the early approval of Horizon’s first Biologics License Application – a key step in our evolution to an innovation-focused biopharma company, developing new medicines for debilitating diseases with few or no treatment options.”

The FDA approval of TEPEZZA comes ahead of the Prescription Drug User Fee Act (PDUFA) goal date of March 8, 2020. The medicine received Priority Review, Orphan Drug, Fast Track and Breakthrough Therapy designations from the FDA.

“The FDA approval of TEPEZZA is momentous for the TED community and has the potential to change the treatment paradigm for TED – providing new hope for people who are living with this horrible, vision-threatening disease,” said Raymond Douglas, M.D., Ph.D., director of the Orbital and Thyroid Eye Disease Program, Cedars-Sinai Medical Center and co-principal investigator of the TEPEZZA Phase 3 confirmatory clinical trial. “Today’s news brings forward a medicine for patients that targets the underlying biology of the disease and has been shown to significantly improve eye bulging and double vision, which are the most debilitating aspects of the disease.”

“TEPEZZA is a much-needed breakthrough for a community of people who have historically had to struggle in pain as their symptoms progress – risking permanent damage to their eyes and making it extremely difficult to go about their daily lives,” said Jeff Todd, president and chief executive officer, Prevent Blindness. “This approval is meaningful to our organization because we are committed to helping patients with vision impairment and those who are at significant risk.”

Horizon will conduct a post-marketing study to evaluate safety in a larger patient population as was discussed at the Dermatologic and Ophthalmic Drugs Advisory Committee (DODAC) FDA Advisory Committee meeting on December 13, 2019, where the committee voted unanimously (12-0) that TEPEZZA demonstrated a positive benefit risk profile. This study will also evaluate retreatment rates relative to how long patients receive the medicine.

As a result of the FDA approval of TEPEZZA, Horizon will make approximately $105 million in milestone payments during the first half of 2020.