FDA approves first treatment for thyroid eye disease

Today, the U.S. Food and Drug Administration (FDA) approved Tepezza (teprotumumab-trbw) for the treatment of adults with thyroid eye disease, a rare condition where the muscles and fatty tissues behind the eye become inflamed, causing the eyes to be pushed forward and bulge outwards (proptosis). Today's approval represents the first drug approved for the treatment of thyroid eye disease.

"Today's approval marks an important milestone for the treatment of thyroid eye disease. Currently, there are very limited treatment options for this potentially debilitating disease. This treatment has the potential to alter the course of the disease, potentially sparing patients from needing multiple invasive surgeries by providing an alternative, nonsurgical treatment option," said Wiley Chambers, M.D., deputy director of the Division of Transplant and Ophthalmology Products in the FDA's Center for Drug Evaluation and Research. "Additionally, thyroid eye disease is a rare disease that impacts a small percentage of the population, and for a variety of reasons, treatments for rare diseases are often unavailable. This approval represents important progress in the approval of effective treatments for rare diseases, such as thyroid eye disease."

Thyroid eye disease is associated with the outward bulging of the eye that can cause a variety of symptoms such as eye pain, double vision, light sensitivity or difficulty closing the eye. This disease impacts a relatively small number of Americans, with more women than men affected. Although this condition impacts relatively few individuals, thyroid eye disease can be incapacitating. For example, the troubling ocular symptoms can lead to the progressive inability of people with thyroid eye disease to perform important daily activities, such as driving or working.

Tepezza was approved based on the results of two studies (Study 1 and 2) consisting of a total of 170 patients with active thyroid eye disease who were randomized to either receive Tepezza or a placebo. Of the patients who were administered Tepezza, 71% in Study 1 and 83% in Study 2 demonstrated a greater than 2 millimeter reduction in proptosis (eye protrusion) as compared to 20% and 10% of subjects who received placebo, respectively.

The most common adverse reactions observed in patients treated with Tepezza are muscle spasm, nausea, alopecia (hair loss), diarrhea, fatigue, hyperglycemia (high blood sugar), hearing loss, dry skin, dysgeusia (altered sense of taste) and headache. Tepezza should not be used if pregnant, and women of child-bearing potential should have their pregnancy status verified prior to beginning treatment and should be counseled on pregnancy prevention during treatment and for 6 months following the last dose of Tepezza.

The FDA granted this application Priority Review, in addition to Fast Track and Breakthrough Therapy Designation. Additionally, Tepezza received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases or conditions. Development of this product was also in part supported by the FDA Orphan Products Grants Program, which provides grants for clinical studies on safety and efficacy of products for use in rare diseases or conditions.

The FDA granted the approval of Tepezza to Horizon Therapeutics Ireland DAC.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The

agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.