FDA approves new treatment for rare hereditary disease

[8/23/2018] The U.S. Food and Drug Administration today approved Takhzyro (lanadelumab), the first monoclonal antibody approved in the U.S. to treat patients 12 years and older with types I and II hereditary angioedema (HAE). HAE is a rare and serious genetic disease that affects people with low levels of and poorly functioning C1-INH proteins in the body. This results in recurrent, unpredictable episodes of severe swelling in different areas of the body, including the stomach, limbs, face and throat.

Takhzyro is a plasma kallikrein inhibitor that is used to prevent swelling attacks from occurring. Takhzyro is a fully human IgG1 monoclonal antibody made in recombinant Chinese Hamster Ovary cells. This approval gives patients and healthcare professionals an additional treatment option.

HAE affects an estimated 1 in 50,000 men and women. Type I is the most common, and accounts for 85 percent of cases. Symptoms of HAE typically begin in childhood and worsen following puberty. Some patients may have many attacks each month, while others will go months without an attack.

FDA based its approval on data from a multicenter, randomized, double-blind, placebo-controlled, parallel-group study in 125 patients with HAE. Patients who received Takhzyro had clinically meaningful and statistically significant reductions in the rate of investigator-confirmed HAE attacks compared to placebo over a 6-month treatment period.

The most common adverse drug reactions in patients taking Takhzyro in clinical trials are injection site reactions, upper respiratory infections, headache, rash, muscle pain, dizziness and diarrhea.

The FDA granted this application **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** and **Breakthrough Therapy (/ForPatients/Approvals/Fast/ucm405397.htm)** designation. Takhzyro also received **Orphan Drug (/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm)** designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

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