FDA Approves First Treatment for Rare Blood Cancer

Elzonris approved for bone marrow disease that can evolve into leukemia.

December 21, 2018 By Food and Drug Administration (FDA)

The U.S. Food and Drug Administration today approved Elzonris (tagraxofusp-erzs) infusion for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and in pediatric patients, two years of age and older.

“Prior to today’s approval, there had been no FDA approved therapies for BPDCN. The standard of care has been intensive chemotherapy followed by bone marrow transplantation. Many patients with BPDCN are unable to tolerate this intensive therapy, so there is an urgent need for alternative treatment options,” said Richard Pazdur, MD, director of the FDA’s Oncology Center of Excellence and acting director of the Office of Hematology and Oncology Products in the FDA’s Center for Drug Evaluation and Research.

BPDCN is an aggressive and rare disease of the bone marrow and blood that can affect multiple organs, including the lymph nodes and the skin. It often presents as leukemia or evolves into acute leukemia. The disease is more common in men than women and in patients 60 years and older.

The efficacy of Elzonris was studied in two cohorts of patients in a single-arm clinical trial. The first trial cohort enrolled 13 patients with untreated BPDCN, and seven patients (54%) achieved complete remission (CR) or CR with a skin abnormality not indicative of active disease (CRc). The second cohort included 15 patients with relapsed or refractory BPDCN. One patient achieved CR and one patient achieved CRc.

Common side effects reported by patients in clinical trials were capillary leak syndrome (fluid and proteins leaking out of tiny blood vessels into surrounding tissues), nausea, fatigue, swelling of legs and hands (peripheral edema), fever (pyrexia), chills and weight increase. Most common laboratory abnormalities were decreases in lymphocytes, albumin, platelets, hemoglobin and calcium, and increases in glucose and liver enzymes (ALT and AST). Health care providers are advised to monitor liver enzyme levels and for signs of intolerance to the infusion. Women who are pregnant or breastfeeding should not take Elzonris because it may cause harm to a developing fetus or newborn baby.

The labeling for Elzonris contains a Boxed Warning to alert health care professionals and patients

about the increased risk of capillary leak syndrome which may be life-threatening or fatal to patients in treatment.

The FDA granted this application Breakthrough Therapy and Priority Reviewdesignation. Elzonris also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Elzonris to Stemline Therapeutics.

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