FDA approves drug to treat rare immune disease

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(HealthDay)—The U.S. Food and Drug Administration on Monday

approved Gamifant (emapalumab-lzsg) for the treatment of primary hemophagocytic lymphohistiocytosis (HLH) in pediatric (as young as newborn) and adult patients who have refractory, recurrent, or progressive disease or intolerance with conventional HLH treatment.

Gamifant is the first drug to be approved specifically for HLH. The drug's efficacy was demonstrated in a clinical trial of 27 pediatric patients with suspected or confirmed primary HLH. Patients had refractory, recurrent, or progressive disease while being treated with conventional HLH therapy or were intolerant of conventional HLH therapy. Patients were a mean of 1 year old. Sixty-three percent of patients treated with Gamifant experienced a response, and 70 percent proceeded to stem cell transplant.

Common side effects reported with Gamifant in clinical trials include infections, hypertension, infusion-related reactions, low potassium, and fever. The FDA advises that patients being treated with Gamifant should not receive any live vaccines and should be tested for latent tuberculosis. The agency also advises health care professionals to closely monitor patients while they are taking Gamifant and to treat them promptly for infections.

"Primary HLH is a rare and life-threatening condition typically affecting children and this approval fills an unmet medical need for these patients," Richard Pazdur, M.D., director of the FDA's Oncology Center of Excellence and acting director of the Office of Hematology and Oncology Products in the FDA Center for Drug Evaluation and Research, said in a statement. "We are committed to continuing to expedite the development and review of therapies that offer meaningful treatment options for patients with rare conditions."

More information: More Information