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[Home](#) > FDA approves first treatment for patients with rare and life-threatening immune disease

Generic Name:

Emapalumab-lzsg

Trade Name:

Gamifant

Company:

Novimmune SA

Notes:

FDA approved [emapalumab-lzsg](#) for the treatment of pediatric (newborn and above) and adult patients with primary hemophagocytic lymphohistiocytosis (HLH) who have refractory, recurrent, or progressive disease or intolerance to conventional HLH therapy. This FDA approval is the first for a drug specifically for HLH.

HLH is a condition in which the body's immune cells do not work properly and start to damage the body's own organs, including the liver, brain, and bone marrow. It can be inherited, which is known as primary or 'familial' HLH. It can also have noninherited causes. People with primary HLH usually develop symptoms within the first months or years of life. Symptoms may include fever, enlarged liver or spleen, and decreased number of blood cells.

Emapalumab-lzsg efficacy was studied in a clinical trial of 27 pediatric patients with suspected or confirmed primary HLH with either refractory, recurrent, or progressive disease during conventional HLH therapy or who were intolerant of conventional HLH therapy. The median age of the patients in the trial was 1 year old.

The study showed that 63% of patients experienced a response, and 70% were able to proceed to stem cell transplant.

Common adverse effects in clinical trials included infections, hypertension, infusion-related reactions, low potassium, and fever.

Patients receiving the agent should not receive any live vaccines and should be tested for latent tuberculosis. Patients should be closely monitored and treated promptly for infections while receiving emapalumab-lzsg.

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