

## PRESS RELEASE

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### US FDA grants Priority Review to Sobi's supplemental Biologics License Application (sBLA) for Gamifant® (emapalumab-lzsg)

Sobi® (STO: SOBI), today announced that the U.S. Food and Drug Administration (FDA) has accepted the supplemental Biologics License Application (sBLA) for Gamifant® (emapalumab-lzsg) for use in adult and paediatric patients with hemophagocytic lymphohistiocytosis (HLH)/macrophage activation syndrome (MAS) in Still's disease with an inadequate response or intolerance to glucocorticoids, or with recurrent MAS. The application was granted Priority Review with a PDUFA date of June 27, 2025.

HLH/MAS, a form of HLH, is a severe complication of rheumatic diseases, occurring most frequently in Still's disease including systemic juvenile idiopathic arthritis (sJIA) and adult-onset Still's disease (AOSD). HLH/MAS is a rare systemic disorder of hyperinflammation with common clinical manifestations such as high persistent fever, elevated ferritin, cytopenias, coagulopathies, and hepatosplenomegaly.

"HLH/MAS in Still's disease is a serious and potentially fatal complication where patients can experience intense hyperinflammation and even multiple organ failure," said Lydia Abad-Franch, MD, MBA, Head of R&D and Chief Medical Officer at Sobi. "There is no approved therapy for HLH/MAS today. Gamifant® (emapalumab-lzsg) selectively neutralizes interferon gamma (IFN-γ), a key driver of hyperinflammation, and if approved, may also help reduce the need for high-dose glucocorticoids in these patients."

The application is based on [results from pooled data from two studies](#) which enrolled a total of 39 patients, the EMERALD ([NCT05001737](#)) and the NI-0501-06 ([NCT03311854](#)) studies. Fifty-three percent of patients had a complete response at Week 8 and 85% had a complete response at any time during the studies. Weekly mean glucocorticoid doses were reduced by 70.1% after 2 weeks of treatment.

Gamifant® (emapalumab-lzsg) a monoclonal antibody that binds and neutralises interferon gamma (IFN-γ), was approved by the FDA in 2018 for the treatment of adult and paediatric (newborn and older) patients with primary HLH with refractory, recurrent, or progressive disease or intolerance with conventional HLH therapy.

#### About macrophage activation syndrome (MAS)

Macrophage activation syndrome (MAS) is a severe complication of rheumatic diseases, most frequently systemic juvenile idiopathic arthritis (sJIA) and adult-onset Still's disease (AOSD). MAS is characterised by fever, hepatosplenomegaly, liver dysfunction, cytopenias, coagulation abnormalities and hyperferritinemia, possibly progressing to multiple organ failure and death. MAS is classified as a form of hemophagocytic lymphohistiocytosis (HLH).

#### About Gamifant® (emapalumab-lzsg)

Gamifant® (emapalumab-lzsg) is an anti-interferon gamma (IFN-γ) monoclonal antibody that binds to and neutralises IFN-γ. In the USA, Gamifant® (emapalumab-lzsg) is the first and only treatment approved by the FDA for the treatment of adult and paediatric (newborn and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy. Primary HLH is a rare syndrome of hyperinflammation that usually occurs within the first year of life and can rapidly become fatal unless diagnosed and treated. The FDA approval is based on data from the phase 2/3 studies (NCT01818492 and NCT02069899). Gamifant® (emapalumab-lzsg) is indicated for administration through intravenous infusion over one hour twice per week until hematopoietic stem cell transplantation (HSCT).

**About Sobi®**

Sobi® is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology, and specialty care, Sobi has approximately 1,800 employees across Europe, North America, the Middle East, Asia, and Australia. In 2024, revenue amounted to SEK 26 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at [sobi.com](https://sobi.com) and LinkedIn.

**Contacts**

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