

Pharmacy Focus: Gamifant®



Key Takeaways¹⁻⁶



Gamifant (emapalumab-lzsg) is a first-in-class therapy for both children and adults with primary hemophagocytic lymphohistiocytosis (HLH) and macrophage activation syndrome (MAS) in Still's disease.



HLH and MAS are rare, life-threatening hyperinflammatory conditions that can quickly result in fatality without treatment.



Gamifant can be used as a bridge to a potential curative allogeneic stem cell transplant for those with HLH or until a patient no longer requires therapy of HLH/MAS in Still's disease.



Costs for Gamifant treatment can easily be millions of dollars per month depending on dosing and individual clinical courses.

Disease Overview¹⁻⁶

Description:

- HLH is a hyper-inflammatory condition marked by the over-activation of white blood cells.
- The symptoms of MAS are similar to those of HLH, including fever, swollen lymph nodes, and enlargement of the liver and spleen. MAS that occurs in Still's disease has a mortality rate of 10% to 22%.

Characteristics: HLH typically has non-specific findings, meaning that symptoms overlap with other diseases and make diagnosis challenging. Symptoms required for HLH diagnosis include fever, splenomegaly (enlarged spleen), and cytopenia (low blood cell counts), among others.

Subtypes & Causes: There are two forms of HLH:

- Primary HLH is genetic and more common in children.
- Secondary (acquired) HLH is commonly triggered by infection or cancer; when this form is caused by an autoimmune disorder, it is called macrophage activation syndrome (MAS, or MAS-HLH).

Severity: May be fatal if not treated.

Occurrence:

- HLH is an extremely rare condition; the estimated incidence of HLH in children younger than 18 years old is one in approximately 100,000.
- Still's disease occurs in less than one per 100,000 people, while MAS occurs in 12% to 14% of patients with Still's disease.



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Treatment⁶

Gamifant® (emapalumab-lzsg) is used for the treatment of adult and pediatric (newborn and older) patients with:

- Primary HLH with refractory, recurrent, or progressive disease or intolerance to conventional HLH therapy.
- HLH/macrophage activation syndrome (MAS) in known or suspected Still's disease, including systemic Juvenile Idiopathic Arthritis (sJIA), with an inadequate response or intolerance to glucocorticoids or with recurrent MAS.

Gamifant®	
Generic Name	emapalumab-lzsg
Manufacturer	Sobi, Inc.
Approval	November 2018
Use	HLH
How It Works	Binds to and neutralizes interferon gamma (IFN-γ), which plays a pivotal role in the hyperinflammation seen in HLH.
Procedure Location	Given as an intravenous injection at a physician's office or hospital.
Timeframe	HLH: Given twice weekly until transplant can be achieved. HLH/MAS in Still's disease: Given every 3 days for 4 doses, then twice per week.
Efficacy	Open-label phase 2/3 clinical trial – N10501-04 (NCT01818492) – looked at 27 pediatric patients with suspected or confirmed primary HLH in whom conventional therapy did not work; treatment with Gamifant resulted in a statistically significant difference in overall response rate in 17 (63%) of the patients.
Estimated Cost Per Month	HLH: Child – ~\$1.2 million / Adult – ~\$2.4 million HLH/MAS in Still's disease or sJIA: Child – ~\$640K / Adult – ~\$1.1 million
Associated Benefit	Medical

*Estimated cost based on average wholesale price for one month of therapy at the maximum recommended dose based on the prescribing information for a 35 kg child and 70 kg adult.

HMConnects™

As part of the HMConnects cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with HMPHarmacyServices@hmig.com.



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MTG-3655 (12/25)

References: ¹George MR. Hemophagocytic lymphohistiocytosis: review of etiologies and management. *J Blood Med*. 2014 Jun 12;5:69-86. ²Rosée PL, Horne A, Hines M, et al. Recommendations for the management of hemophagocytic lymphohistiocytosis in adults. *Blood*. 2019;133(23):2465–2477. ³Morimoto A, Nakazawa Y, Ishii E. Hemophagocytic lymphohistiocytosis: pathogenesis, diagnosis, and management. *Pediatr Int*. 2016;58(9):817-825. ⁴Efthimiou P, Kadavath S, Mehta B. Life-threatening complications of adult-onset Still's disease. *Clin Rheumatol*. 2014 Mar;33(3):305-14. ⁵Bhargava J, Panginikkod S. Still Disease. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK538345/>. ⁶Gamifant package insert, Waltham, Massachusetts: Sobi, Inc.; June 2025.

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