PRESS RELEASE

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New data for emapalumab in the treatment of macrophage activation syndrome to be presented at the ACR conference

Sobi® (STO: SOBI) today announced that new research showing the effect of emapalumab in patients with macrophage activation syndrome (MAS) in Still's disease, including systemic juvenile idiopathic arthritis (sJIA) and adult-onset still's disease (AOSD) who had an inadequate response to high-dose glucocorticoids (GCs) will be presented at the American College of Rheumatology (ACR) Convergence in Washington D.C. The data were pooled from two open-label, single-arm interventional studies, the phase 3 NI-0501-14 study [EMERALD; NCT05001737] and NI-0501-06 [NCT03311854], including 39 patients with MAS.

Key results:

- **Complete response at week 8:** At week 8, 21 patients (53.8%) achieved a complete response (95% confidence interval (CI): 37.2–69.9%).
- **Complete response at any time:** 33 patients (85%) achieved a complete response at any time during the studies.
- **Glucocorticoid tapering:** Weekly mean glucocorticoid doses were reduced by 70.1% after 2 weeks of treatment. By week 8, glucocorticoids were tapered to a clinically meaningful dose of ≤1 mg/kg/day in 28 patients (72%) and to ≤0.5 mg/kg/day in 17 patients (44%).
- **Safety:** No new safety concerns were identified during the studies.

"MAS in Still's disease is a severe condition characterised by intense hyperinflammation and multiple organ failure. Those affected, including young children, often experience high fevers, liver and spleen enlargement, severe cytopenias, and neurological symptoms," said Lydia Abad-Franch, MD, Head of Research & Development and Medical Affairs and Chief Medical Officer at Sobi. "The results to be presented at ACR indicate that emapalumab could potentially provide a new therapeutic option, offering effective control of MAS symptoms and possibly reducing the need for high-dose glucocorticoids. This represents an important development in the treatment landscape for MAS."

Additional key results:

- **Overall response (OR) by week 8:** 32 patients (82.4%) achieved an overall response by week 8. OR was observed as early as day 5, with a median time to first OR of 2.3 weeks.
- **Clinical MAS remission:** 32 patients (82.1%) achieved investigator-assessed clinical MAS remission at any time , with a median time to clinical remission of 3.3 weeks.
- **Survival rate:** 37 patients (94.9%) were alive at week 8.

Emapalumab is a monoclonal antibody that neutralises interferon gamma (IFNγ), a key cytokine which contributes to the inflammation and tissue damage seen in MAS. The purpose of these two prospective studies is to assess the efficacy and safety of emapalumab in controlling MAS in patients with Still's disease, including sJIA and AOSD, who had an inadequate response to high-dose glucocorticoids. In May 2024, the US FDA granted emapalumab fast track designation as a potential therapeutic option in patient with MAS. Sobi plans to submit an sBLA for this indication to the FDA in 2024.

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The data will be presented by Alexei A. Grom, MD, Professor of Paediatrics, Research Director Division of Rheumatology, Cincinnati Children's Hospital Medical Centre, in a session entitled "Efficacy and Safety of emapalumab in children and adults with macrophage activation syndrome (MAS) in Still's disease: Results from a phase 3 study and a pooled analysis of two prospective trials" on Tuesday, 19 November at ACR Convergence.

About macrophage activation syndrome (MAS)
Macrophage activation syndrome (MAS) is a severe complication of rheumatic diseases, most frequently systemic juvenile idiopathic arthritis (sJIA) – a rare systemic disorder of auto-inflammatory nature with common clinical manifestations such as daily spiking fever, typical transient cutaneous rash, arthritis, lymphadenopathy, hepatosplenomegaly and serositis. MAS is characterised by fever, hepatosplenomegaly, liver dysfunction, cytopenias, coagulation abnormalities and hyperferritinaemia, possibly progressing to multiple organ failure and death. MAS is classified as a secondary form of haemophagocytic lymphohistiocytosis (HLH).

About emapalumab
Emapalumab (Gamifant®) is an anti-interferon gamma (IFNγ) monoclonal antibody that binds to and neutralises IFNγ. In the USA, emapalumab is indicated for the treatment of adult and paediatric (newborn and older) patients with primary haemophagocytic 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 is indicated for administration through intravenous infusion over one hour twice per week until haematopoietic 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 2023, revenue amounted to SEK 22.1 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

Contacts
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