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New data demonstrates impact of emapalumab in patients with macrophage activation syndrome (MAS)

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) announces that new research demonstrating the effects of emapalumab in patients with macrophage activation syndrome (MAS), a form of secondary haemophagocytic lymphohistiocytosis (HLH) complicating systemic juvenile idiopathic arthritis (sJIA), was presented at the European League Against Rheumatism (EULAR)/ Paediatric Rheumatology European Society (PReS) Scientific Congress in Madrid.

The study showed that treatment with emapalumab led to rapid neutralisation of interferon gamma (IFNγ) and a complete response in all patients who participated in the study. Furthermore, emapalumab demonstrated a favourable safety profile[1]. Emapalumab has previously been shown to induce rapid and sustained responses in patients with primary HLH.

Emapalumab is FDA-approved for paediatric (newborn and older) and adult patients with primary HLH with refractory, recurrent or progressive disease or intolerance to conventional HLH therapy.

The data was also recognised with the Gold PReS KOURIR Award during the conference.

MAS is a rare, life-threatening condition characterised by uncontrolled hyperinflammation which may develop on a background of rheumatic diseases such as sJIA. It is classified as a secondary form of HLH and is caused by excessive activation and expansion of T cells and macrophages. A vast body of evidence has been accumulated which points to uncontrolled overproduction of IFNγ as a major driver of hyperinflammation and hypercytokinaemia in diseases such as MAS and HLH.

“It is extremely encouraging to see a complete response in the first six patients treated with emapalumab, particularly considering that MAS is a serious and potentially fatal complication, and that all of these patients had failed other treatments,” said Dr. Fabrizio De Benedetti, Head of the Division of Paediatric Rheumatology and Head of the Laboratory of ImmunoRheumatology, Ospedale Pediatrico Bambino Gesù, Rome, Italy.

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[1] **Emapalumab, an interferon gamma (IFN-Y)-blocking monoclonal antibody, in patients with macrophage activation syndrome (MAS) complicating systemic juvenile idiopathic arthritis (sJIA)**
Fabrizio De Benedetti, Paul Brogan, Alexei Grom, Pierre Quartier Dit Maire, Rayfel Schneider, Kathy De Graaf, Philippe Jacqmin, Maria Ballabio, Cristina de Min

The new data represents the results of an on-going international pilot study, in which six MAS patients with a background of sJIA and inadequate response to high-dose intravenous glucocorticoids received emapalumab according to the prescribed dose regimen required per protocol.

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 the study is to assess the drug's pharmacokinetics, efficacy and safety for treatment of MAS and to confirm the proposed dose regimen.

The data was presented in a session entitled "Adults are just grown up children! Discuss" on Thursday, 13 June, by Dr. De Benedetti. Details of the abstract can be accessed at the EULAR website: http://scientific.sparx-ip.net/archiveeular/?c=a&view=3&item=2019OP0204

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 is a monoclonal antibody (mAb) that binds to and neutralises interferon gamma (IFNγ). In the US, emapalumab is indicated for paediatric (newborn and older) and adult primary haemophagocytic lymphohistiocytosis (HLH) patients with refractory, recurrent or progressive disease, or intolerance to standard-of-care HLH therapy, and is marketed under the name Gamifant. Emapalumab is the first and only medicine approved in the US for primary HLH, 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). Emapalumab is indicated to be administered through intravenous (IV) infusion over one hour twice per week until haematopoietic stem cell transplant (HSCT). Visit www.gamifant.com for more information, including full US prescribing Information.

Emapalumab was developed and submitted for approval to the FDA by Novimmune, a privately held Swiss biopharmaceutical company focused on discovering and developing antibody-based drugs targeted for the treatment of inflammatory diseases, immune-related disorders and cancer. Sobi acquired the global rights to emapalumab from Novimmune through an exclusive licensing agreement announced in July 2018, which is now superseded by the acquisition of emapalumab and related assets announced on 12 June 2019, subject to customary closing conditions.

About Sobi™
At Sobi, we are transforming the lives of people affected by rare diseases. As a specialised international biopharmaceutical company, we provide sustainable access to innovative therapies in the areas of haematology, immunology and specialty care. We bring something rare to rare diseases – a belief in the strength of focus, the power of agility and the potential of the people we are dedicated to serving. The hard work and dedication of our

approximately 1050 employees around the globe has been instrumental in our success across Europe, North America, the Middle East, Russia and North Africa, leading to total revenues of SEK 9.1 billion in 2018. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

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