PRESS RELEASE

Stockholm, Sweden, 7 May 2020

Results from pivotal phase 2/3 study of emapalumab in patients with primary HLH published in *New England Journal of Medicine*

Sobi™ announced today that the results from the pivotal phase 2/3 study evaluating the efficacy and safety of emapalumab in patients with primary haemophagocytic lymphohistiocytosis (HLH) were published in the *New England Journal of Medicine* on 7 May 2020. Emapalumab is the first therapy approved by the US Food & Drug Administration (FDA) for primary HLH and is under review by the European Medicines Agency (EMA).

Primary HLH is a rare syndrome that typically presents in infancy but can also be seen in adults and is associated with high morbidity and mortality. This life-threatening disease is characterised by immune dysregulation and uncontrolled hyperinflammation. The treatment objective is to suppress the hyperinflammation and control the acute features of the disease in order to successfully bring patients to haematopoietic stem cell transplantation (HSCT).

“The publication of the results in this highly respected medical journal is a testament to the medical importance of the emapalumab findings for patients with primary HLH,” says Milan Zdravkovic, Head of Research & Development and Chief Medical Officer at Sobi. “The results further advance our understanding of primary HLH and the role of interferon gamma in its pathogenesis. Our hope is to contribute to the improvement of care and treatment for patients suffering from this potentially fatal disease.”

The results with emapalumab in primary HLH published in the *New England Journal of Medicine* highlight the overall response rate of 63 percent in previously treated patients at the end of up to 8 weeks of treatment (compared to the pre-specified null hypothesis of 40 percent (p=0.02)). In the previously treated group, 70 percent of patients were able to proceed to transplantation. The most commonly reported adverse reactions (≥ 20 per cent) were infections, hypertension, infusion-related reactions and fever.

Michael Jordan, Professor of Pediatrics at the Cincinnati Children’s Hospital Medical Center in the US and coordinating Principal Investigator of the study (US), confirms the importance of making advances in finding new therapies for HLH and emphasises the importance of the publication of the results: “The findings from the study are encouraging for those affected by this devastating disease.”

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Professor Franco Locatelli, Head of the Department of Onco-Haematology, Bambino Gesù Children's Hospital IRCCS, Sapienza University of Rome, Italy, and coordinating Principal Investigator (EU), adds: "Emapalumab represents a prototype model molecularly targeted therapy and an important step towards improving outcomes for this severe and life-threatening disease."

This pivotal clinical study is the first study in primary HLH to prospectively assess and report treatment responses using predefined comprehensive objective clinical and laboratory criteria. Preclinical data have shown the central role of interferon gamma (IFNγ) in the pathogenesis of this disease[1]. Emapalumab is a monoclonal antibody that binds to and neutralises IFNγ. It was approved by the US Food & Drug Administration (FDA) on the basis of this clinical study for the treatment of primary HLH in adult and paediatric (newborn and older) patients with refractory, recurrent or progressive disease, or intolerance to conventional HLH therapy, and received Breakthrough Designation prior to review.

About emapalumab

Emapalumab is a monoclonal antibody 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 conventional HLH therapy. 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 for administration through intravenous infusion over one hour twice per week until haematopoietic stem cell transplantation (HSCT). For more information please see www.gamifant.com including the full US Prescribing Information. Emapalumab is under review for primary HLH by the European Medicines Agency (EMA).

About Sobi™

Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,400 people across Europe, North America, the Middle East, Russia and North Africa. In 2019, Sobi's revenues amounted to SEK 14.2 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at sobi.com.

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1. Jordan et al. Blood 2004;104:735-43.