PRESS RELEASE

Stockholm, Sweden, 26 February 2019

New emapalumab data presented at Transplantation and Cellular Therapy (TCT) Meeting highlight post-transplant treatment outcomes in primary HLH

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Novimmune SA announced the presentation of late-breaking data from the phase 2/3 clinical study of emapalumab-lzsg in primary haemophagocytic lymphohistiocytosis (HLH) at the 2019 Transplantation and Cellular Therapy (TCT) Meetings, February 20-24 in Houston, Texas. The data examine the outcomes of primary HLH patients who were treated with emapalumab and proceeded to haematopoietic stem cell transplantation (HSCT).

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 goal of conventional therapy for HLH is to bring the hyperinflammatory emergency under control, usually with a combination of steroids and chemotherapy, and to prepare for HSCT, which is the only cure.

Emapalumab is an interferon gamma (IFNγ)-blocking antibody approved by the US FDA as Gamifant® for the treatment of paediatric (newborn and older) and adult patients with primary HLH with refractory, recurrent or progressive disease or intolerance to conventional HLH therapy.

“In this study, the majority of these very sick patients who were treated with emapalumab were able to reach transplant, engraft and survive,” said Michael Jordan MD, a physician-scientist in the division of Bone Marrow Transplantation and Immune Deficiency at Cincinnati Children's Hospital Medical Center HLH Center of Excellence, and Primary Investigator in the emapalumab clinical study. “These are encouraging outcomes given the median survival for patients with primary HLH is less than two months without treatment, and many fail conventional therapy.”

“We are encouraged by continuing to see clinical data that shows the potential of emapalumab in primary HLH,” said Cristina de Min, Chief Medical Officer at Novimmune, and Milan Zdravkovic, Chief Medical Officer and Head of Research & Development at Sobi.

In the phase 2/3 study, most patients treated with emapalumab proceeded to HSCT: 64.7 per cent of all patients treated and 70.4 per cent of patients treated after failing prior conventional HLH therapy. The median time to transplant was 100 days for the full cohort and 83 days for the cohort failing prior conventional HLH therapy. Stem cell sources included bone marrow, primarily, followed by peripheral blood, then cord blood. Most patients successfully underwent engraftment (86.4 per cent of the full cohort and 89.5 per cent of the cohort failing prior conventional HLH therapy).

Ultimately, 90.9 per cent of the full cohort survived post-HSCT, as did 89.5 per cent of the cohort failing prior conventional HLH therapy.

The data were presented in a late-breaking abstract presentation entitled "*Post-Transplant Outcomes of Children with Primary Hemophagocytic Lymphohistiocytosis Given Emapalumab to Control Disease*" on Sunday, 24 February, by Dr. Jordan.
The global, multicentre, open-label, single-arm, phase 2/3 clinical study enrolled 34 patients with a diagnosis of primary HLH. Of the 34 patients enrolled, 27 had failed prior conventional HLH therapy. The data cut-off applied is 20 July 2017.

Details of the abstract can be accessed at the TCT website:
https://tct.confex.com/tct/2019/meetingapp.cgi/Paper/13801

About primary haemophagocytic lymphohistiocytosis (HLH)
Primary haemophagocytic lymphohistiocytosis (HLH) is a rare, rapidly progressive, often-fatal syndrome of hyperinflammation in which massive hyperproduction of interferon gamma (IFNγ) is thought to drive immune system hyperactivation, ultimately leading to organ failures. It is estimated that fewer than 100 cases of primary HLH are diagnosed each year in the US, but this is believed to represent underdiagnosis. Diagnosis is challenging due to the variability in signs and symptoms, which may include fevers, swelling of the liver and spleen, severe low red and white blood cell counts, bleeding disorders, infections, neurological symptoms, organ dysfunction and organ failure. Primary HLH can rapidly become fatal if left untreated, with median survival of less than two months. The immediate goal of treatment is to quickly control the hyperinflammation and to prepare for haematopoietic stem-cell transplant. The current conventional treatment prior to transplant includes steroids and chemotherapy and is not specifically approved to treat primary 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. 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. Sobi acquired the global rights to emapalumab from Novimmune through an exclusive licensing agreement announced in July 2018.

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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.

About Novimmune SA

Novimmune SA is 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. Founded in 1998 by the renowned immunologist Professor Bernard Mach, Novimmune has more than 150 employees and operates in two sites in Geneva and Basel (Switzerland). Since its foundation, Novimmune has built a significant R&D pipeline of drug candidates, of which emapalumab is the most advanced. The development program of Gamifant was supported by a FP7 grant from the European Commission (FIGHT HLH). Novimmune has also developed a bispecific antibody generation platform designed to streamline the identification, production and characterization of fully-human bispecific antibodies. More information is available at www.novimmune.com.

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