PRESS RELEASE

Stockholm, Sweden, and Geneva, Switzerland, 4 December 2018

Data presented at ASH support emapalumab as an innovative, targeted therapeutic option for primary HLH

Swedish Orphan Biovitrum AB (publ) (Sobi™) and Novimmune SA are presenting data from the pivotal phase 2/3 clinical study of emapalumab-lzsg in primary haemophagocytic lymphohistiocytosis (HLH) as part of the late-breaking abstract session at the 60th Annual Meeting of the American Society of Hematology (ASH), taking place 1-4 December 2018, in San Diego.

“Primary HLH is an aggressive hyper-inflammatory syndrome that can quickly become fatal if not treated. This study demonstrated that emapalumab treatment induced rapid and sustained responses, helping these fragile and often very young patients control HLH activity and reach stem cell transplant, which is the only cure for this devastating disease, potentially without the side effects of prolonged chemotherapy. Further, the 90 per cent survival post-transplant in this study is very encouraging, particularly given the high morbidity and mortality associated with primary HLH,” said first author and presenter of the late-breaking abstract Professor Franco Locatelli, Head of the Department of Onco-Haematology, Bambino Gesù Children’s Hospital IRCCS, Rome, Italy.

The trial achieved its primary endpoint, with 64.7 per cent of all patients treated (22 of 34; p=0.0031) and 63 per cent of the patients who had failed prior conventional HLH therapy (17 of 27; p=0.0134) demonstrating an overall response at the end of treatment, defined as achievement of either a complete or partial response, or HLH improvement. Across both cohorts, the overall response rate (ORR) was significantly higher than the pre-specified null hypothesis of 40 per cent. The median time to response was eight days, and patients remained in response for a median of 75 per cent of the time.

The majority of all patients treated – 64.7 per cent (22 of 34) of the full patient group and 70.4 per cent (19 of 27) of the patients who had failed prior conventional HLH therapy – proceeded to haematopoietic stem cell transplantation (HSCT), with 90.9 per cent (20 of 22) of the full group and 89.5 per cent (17 of 19) of the subgroup surviving post HSCT. The most common adverse reactions reported during the study were infections (56 per cent), hypertension (41 per cent), infusion-related reactions (27 per cent) and fever (24 per cent).

“We are very encouraged by the data being presented at ASH on emapalumab in primary HLH, and we look forward to investigating emapalumab in additional diseases for which interferon gamma is considered pathogenic,” said Cristina de Min, Chief Medical Officer at Novimmune, and Milan Zdravkovic, Chief Medical Officer and Head of Research & Development at Sobi.

The data will be presented in an abstract titled "*Safety and efficacy of emapalumab in pediatric patients with primary hemophagocytic lymphohistiocytosis*" on Tuesday, 4 December 2018, at 08:45 Pacific Time during the 07:30-09:15 late-breaker session, by Dr. Locatelli.

Emapalumab is an interferon gamma (IFNγ) blocking antibody approved by the U.S. FDA as Gamifant® for the treatment of paediatric (new born and older) and adult patients with primary HLH with refractory, recurrent or progressive disease or intolerance to conventional HLH therapy. Primary HLH is an ultra-rare, genetic, life-threatening syndrome characterised by hyper-inflammation that is thought to be driven by high production of IFNγ.

The global, multicentre, open-label, single-arm, pivotal phase 2/3 clinical study enrolled 34 patients with a diagnosis of primary HLH based on genetic confirmation, family history, or the presence of ≥5 of the 8 HLH-2004 diagnostic criteria. Patients received emapalumab intravenously every 3-4 days, starting at 1 mg/kg and increasing as needed up to 10 mg/kg. Emapalumab was administered concomitantly with dexamethasone, which could be tapered during the study. Average treatment duration was 8 weeks, with possible shortening to a minimum of 4 weeks and possible extension up to haematopoietic stem cell transplantation (HSCT) as needed. Of the 34 patients enrolled, 27 had failed prior conventional HLH therapy. Details on the abstract can be accessed at the ASH website: https://ash.confex.com/ash/2018/webprogram/Paper120810.html

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About primary haemophagocytic lymphohistiocytosis (HLH)

Primary haemophagocytic lymphohistiocytosis (HLH) is an ultra-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 under diagnosis. 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 are 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 (new born 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, an ultra-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 pivotal phase 2/3 study (NCT01818492). 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.

About Sobi™

Sobi™ is an international speciality healthcare company dedicated to rare diseases. Our vision is to be recognised as a global leader in providing access to innovative treatments that transform lives for individuals with rare diseases. The product portfolio is primarily focused on treatments in Haemophilia and Specialty Care. Partnering in the development and commercialisation of products in specialty care is a key element of our strategy. Sobi has pioneered in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2017, Sobi had total revenues of SEK 6.5 billion and approximately 850 employees. The share (STO:SOBI) is listed on Nasdaq Stockholm. More information is available at www.sobi.com.

About Novimmune

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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