PRESS RELEASE

Stockholm, Sweden, 24 July 2020

Sobi will file for a re-examination of emapalumab in Europe following negative opinion by CHMP

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) today announced that the Committee for Medicinal Products for Human use (CHMP) has adopted a negative opinion recommending a refusal of the marketing authorisation for emapalumab for the treatment of primary haemophagocytic lymphohistiocytosis (HLH) in children under 18 years of age in Europe. Given the significant unmet medical need that emapalumab addresses in patients with primary HLH with no approved treatments in Europe, Sobi will be requesting a re-examination by the CHMP with an expected opinion by end of year 2020.

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. In spite of some treatment advances, there continues to be a very high unmet medical need in particular in patients that have failed conventional therapy as there are no approved treatment options outside the US. In the US, emapalumab is the first therapy approved by the US Food & Drug Administration (FDA) for primary HLH. Over 100 patients have been treated in the US and the benefit/risk profile continues to be favourable.

“Emapalumab has demonstrated a positive benefit/risk profile in primary HLH in a post-approval real life setting in the US since the FDA approval in 2018. The product has been able to make a substantial difference for a very vulnerable group of patients in the US. We are proud of having made a significant contribution with our product in the primary HLH indication and we are gratified by the recent academic validation of our work via publication in the New England Journal of Medicine. During the last years our team has gained a lot of experience in this rather complex disease area. We will do our utmost to share these insights and address the open questions by CHMP during the re-examination with a view to secure access for primary HLH in children to this treatment in Europe”, says Guido Oelkers, CEO and President of Sobi.

HLH is a rare disease but with a large unmet medical need globally. The most important markets based on number of patients for both primary and secondary HLH are China followed by the US, Europe and Japan. In addition to HLH, Sobi will initiate clinical studies with emapalumab for potential indications such as pre-emptive treatment of patients with risk factors of HSCT acute graft failure which will further expand the patient population and market potential for emapalumab. Sobi’s earlier communicated estimated peak sales target for emapalumab beyond USD 500 million remains unchanged regardless of an approval in Europe.

Professor Franco Locatelli, Principal Investigator in the EU says “In my role as Principal Investigator of the NI-0501-04/05 studies in Europe I was significantly surprised about the EMA decision not to approve emapalumab for children with primary HLH who failed or are intolerant to front-line therapy. I had the privilege to observe that this monoclonal antibody, targeting the main cytokine involved in the disease pathophysiology, was well tolerated and effective in a large proportion of the patients, representing a model of precision medicine. While US children have since almost 2 years the possibility to be treated with this novel, safe, highly effective and targeted therapy, the EMA decision paves the way for migratory health flows towards non-European Centers that can grant this treatment.”

Professor Michael Jordan, Principal Investigator in the US confirms “The NI-0501-04/05 studies have demonstrated that emapalumab has clear therapeutic activity in primary HLH and have validated interferon gamma as a key target in these patients. These studies have also demonstrated that this unique and targeted approach to therapy has a very favorable safety profile. I am grateful for the opportunity to help lead these trials which were conducted with the greatest rigor and transparency, far exceeding that of any trial to date in this very challenging patient population. The worldwide team of collaborators, including physicians at many centers in the US and Europe, as well as individuals at Sobi, should be proud of this ground-breaking achievement. I believe that emapalumab will benefit patients around the world with HLH, especially as we continue to learn how to best apply this unique drug in patients with HLH.“

Recently, the results from the pivotal study evaluating the efficacy and safety of emapalumab in patients with primary HLH were published in one of the highest-ranking medical journals, *New England Journal of Medicine*.

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.

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 www.sobi.com.

This information is information that Swedish Orphan Biovitrum AB (publ) is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of Linda Holmström, Corporate Communication and Investor Relations, at 14:00 CEST on 24 July 2020.

Swedish Orphan Biovitrum AB (publ)
Postal address SE-112 76 Stockholm, Sweden
Phone: +46 8 697 20 00 | www.sobi.com

For more information please contact
Paula Treutiger, Head of Communication & Investor Relations
+ 46 733 666 599
paula.treutiger@sobi.com

Linda Holmström, Corporate Communication & Investor Relations
+ 46 708 734 095
linda.holmstrom@sobi.com