PRESS RELEASE

Stockholm, Sweden, 13 November 2020

The CHMP has adopted a negative opinion for emapalumab in Europe for the treatment of primary HLH

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 (pHLH) in children under 18 years of age in Europe. The negative opinion was given after the re-examination requested by Sobi after the initial opinon in July 2020.

"This recommendation by the CHMP is disappointing given the significant unmet medical need which exists for patients with pHLH who have no approved therapies in Europe. During the re-examination we worked extensively with physicians and patients and were able to resolve some but not all of the concerns raised by EMA," said Ravi Rao, Head of R&D and Chief Medical Officer at Sobi. "We are confident about the clinical profile of emapalumab and our focus is now on increasing access for patients in other regions and developing new indications for this medicine.

About primary HLH

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.

About emapalumab

Emapalumab is a monoclonal antibody that binds to and neutralises interferon gamma (IFNγ). In the US, emapalumab is indicated for the treatment of adult and paediatric (new-born and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy. 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 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. In September 2020, emapalumab received Orphan Drug Designation (ODD) by the FDA for prevention of graft failure following haematopoietic stem cell transplantation.

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 13:00 CET on 13 November 2020.

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