PRESS RELEASE

Stockholm, Sweden, 5 December 2019

Data to be highlighted at the American Society of Hematology 2019 Annual Meeting

Sobi™ and its wholly owned subsidiary Dova Pharmaceuticals, will present new data from studies with avatrombopag for the treatment of thrombocytopenia in adult patients with chronic liver disease (CLD) as well as patients with chronic immune thrombocytopenia (ITP) at the 61st Annual Meeting of the American Society of Hematology (ASH) taking place in Orlando, 7-10 December. In addition, final data from the phase 1/2 repeat dose study with BIVV001 (rFVIIIFc-VWF-XTEN) in severe haemophilia A will be highlighted in a presentation as well as the adaptive design of a study evaluating emapalumab in adults with secondary haemophagocytic lymphohistiocytosis (HLH).

Results from a study to evaluate the relative cost-effectiveness of avatrombopag in CLD compared with platelet transfusion or treatment with lusutrombopag, showed that the use of avatrombopag is a practical strategy compared with the cost of both platelet transfusion and lusutrombopag, as it saves cost and reduces the need for prophylactic platelet transfusions.

Results from an online survey highlighting disease burden and impact for patients with immune thrombocytopenia demonstrated the complexity of ITP and the effects of fatigue, bleeding and treatment on the lives of the patients.

Additional analyses from the phase 3 study – core and extension phase - with avatrombopag for the treatment of ITP confirm the long-term response rates and efficacy of treatment with avatrombopag in this indication.

Final data from a phase 1/2 study of BIVV001 evaluating the safety and pharmacokinetics of repeated dosing in people with severe Haemophilia A will be shared in an oral presentation. BIVV001 is the first investigational von Willebrand (VWF)-independent factor VIII therapy that is designed to provide high sustained factor activity and extend protection from bleeds with once weekly dosing for people with haemophilia A. BIVV001 is being developed in collaboration with Sanofi.

The adaptive design of a study evaluating the efficacy, safety and pharmacokinetics of emapalumab in adult patients with secondary HLH will be presented. The primary efficacy outcome is to evaluate overall response after 4 weeks. The initial phase will enrol 10 adult patients and the study is expected to open later this year.

Sobi supports an independent CME Symposium through an independent educational grant to Physicians' Education Resource (PER) entitled Primary HLH: Diagnosis, Management, and Treatment in the 21st Century. The symposium will provide up-to-date information on the diagnosis, pathobiology, and treatment of primary HLH. The latest clinical data and ongoing trials with the potential to impact the care of patients with HLH will also be reviewed.

Abstracts and symposiums:

- Cost-effectiveness of avatrombopag for the treatment of thrombocytopenia in patients with chronic liver disease. Sunday, 8 December, 2019. Poster #3454.
- Long-term response rates in patients with chronic immune thrombocytopenia treated with avatrombopag: Additional analyses from a phase 3 study and its extension phase. Sunday, 8 December, 2019. Poster #2356.
- Platelet response to avatrombopag in patients with chronic immune thrombocytopenia: Additional analyses from a phase 3 study and its extension: Saturday, 7 December, 2019. Poster #1071.
- Phase 1 repeat dosing with BIVV001 (rFVIIIFc-VWF-XTEN): The first investigational factor VIII product to break through the von Willebrand factor–imposed half-life ceiling. Monday, 9 December2019, 10:30 AM ET. Oral presentation #625. BIVV001 is developed in collaboration with Sanofi.
- An adaptive design study to evaluate the efficacy, safety and pharmacokinetics of emapalumab in adult patients with non-primary hemophagocytic lymphohistiocytosis. Sunday, 8 December, 2019. Poster #2333.
- Primary HLH: Diagnosis, Management, and Treatment in the 21st Century. Satellite Symposia. Friday, 6 December, 2019.

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 1,300 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 BIVV001

BIVV001 (rFVIIIFc-VWF-XTEN) is a novel and investigational recombinant factor VIII therapy that is designed to extend protection from bleeds with once-weekly prophylactic dosing for people with hemophilia A. BIVV001 builds on the innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN polypeptides to potentially extend its time in circulation. It is the first therapy that has been shown to break through the von Willebrand factor ceiling, which is believed to impose a half-life limitation on current factor VIII therapies. BIVV001 was granted orphan drug designation by the US Food and Drug Administration in August 2017 and the European Commission in June 2019.

About avatrombopag
Doptelet® is an oral thrombopoietin (TPO) receptor agonist administered with food. Doptelet is approved by both the United States Food and Drug Administration (FDA) and European Medicines Agency (EMA) for treatment of thrombocytopenia (low platelet counts) in adult patients with chronic liver disease (CLD) who are scheduled to undergo a procedure. In June 2019, Doptelet was approved for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment by FDA. Chronic ITP is a rare autoimmune bleeding disorder characterised by low number of platelets, affecting approximately 60,000 adults in the United States.

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 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 to be administered through intravenous (IV) infusion over one hour twice per week until haematopoietic stem cell transplant (HSCT).

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