PRESS RELEASE

Stockholm, Sweden, 12 March 2021

First patient dosed in phase 3 study with avatrombopag for treatment of ITP in children

Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) today announces that the first patient has been dosed with avatrombopag, a thrombopoietin receptor agonist (TPO-RA), for treatment of immune thrombocytopenia (ITP) in pediatric patients. This phase 3 study, the first the evaluate avatrombopag in children, will evaluate efficacy, safety, and pharmacokinetics of avatrombopag in the treatment of pediatric subjects with ITP.

"We are excited to announce the start of this important study," says Ravi Rao, Head of Research & Development at Sobi. "Our hope is to provide a new treatment option for children with ITP with the potential to decrease the treatment burden that impacts their daily lives."

ITP is an autoimmune disorder that occurs when the body attacks its own platelets and destroys them too quickly. In children with ITP, the body is producing platelets normally but destroying them too quickly, with platelets surviving only a few hours instead of the normal seven to 10 days. The result is a low platelet count in the blood and symptoms such as petechiae, easy bruising, and bleeding events. Spontaneous remission is common in the pediatric population, however 10-20 per cent of children will develop chronic ITP[1].

Symptoms in children with ITP vary but sporadic and unpredictable bleedings along with low platelet counts can lead to anxiety among both the childen and parents. In children with more severe ITP, school attendance and participation in athletic activities have been shown to decrease. Treatment decisions for patients, families and clinicians are challenging given the administration requirements and associated toxicities of the available treatments and an important unmet medical need for new treatment options still remains[2].

About AVA-PED-301 Study

AVA-PED-301 is a phase 3 study evaluating the efficacy, safety, and pharmacokinetics of avatrombopag in the treatment of pediatric subjects with ITP. Eligible pediatric subjects with platelet counts $<30\times10^9$/L will enter a 12-week double-blind phase followed by an open-label extension phase up to 2 years in duration. This study will enroll a total of 72 pediatric subjects (aged ≥1 to <18 years of age) in 9 countries. Subjects will be randomized to blinded therapy of either avatrombopag or placebo in a 3:1 ratio. Enrollment into the study will be staggered by descending age cohort, with a safety and pharmacokinetic review conducted before opening the next cohort for enrolment.

About Doptelet® (avatrombopag)

Doptelet is an orally administered thrombopoietin receptor agonist (TPO-RA) that mimics the biologic effects of TPO in stimulating the development and maturation of megakaryocytes, resulting in increased platelet count. It is approved by both the US Food & Drug

Administration (FDA) for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a procedure and by the European Medicines Agency (EMA) for the treatment of severe thrombocytopenia in adult patients with chronic liver disease (CLD) who are scheduled to undergo an invasive procedure. Doptelet is also approved by the FDA and EMA for the treatment of primary chronic immune thrombocytopenia (ITP) in adult patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Chronic ITP is a rare autoimmune bleeding disorder characterised by a low number of platelets. The incidence of primary ITP in adults is 3.3/100 000 adults per year with a prevalence of 9.5 per 100 000 adults[3].

About immune thrombocytopenia (ITP) in children
Immune thrombocytopenia is an autoimmune disorder characterised by low numbers of platelets, leading to bruising and an increased risk of bleeding. The incidence of primary ITP in children is 2 to 6 per 100 000 children per year[3]. The disorder is considered chronic when symptoms last more than 12 months. These patients have usually relapsed after various treatments and generally require treatment to reduce the risk of clinically significant bleeding.

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 1,500 people across Europe, North America, the Middle East, Asia and North Africa. In 2020, Sobi's revenue amounted to SEK 15.3 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

For more information, please contact

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

Maria Kruse, Corporate Communication & Investor Relations
+ 46 767 248 830
maria.kruse@sobi.com

1. Terrell DR, Beebe LA, Vesely SK, Neas BR, Segal JB, George JN. The incidence of immune thrombocytopenic purpura in children and adults: a critical review of published reports. Am J Hematol. 2010;85(3):174-180.
2. Nichola Cooper, Douglas B. Cines. The child with immune thrombocytopenia: to treat or not to treat, is that still the question?. Haematologica 2019;104(11):2132-2134.
3. Lambert et al. Blood 2017