PRESS RELEASE

Stockholm, 25 March 2024

Sobi announces positive results from phase 3 study of Doptelet® for treatment of children and adolescents with ITP

- *The primary endpoint was met, confirming the efficacy and safety of* avatrombopag (Doptelet®) *in treating children and adolescents with ITP*
- *avatrombopag is widely approved for the treatment of adults with ITP; AVA-PED-301 data will support global regulatory filings to expand to children and adolescents with ITP*

Sobi® today announced positive results from the AVA-PED-301 study (NCT04516967), evaluating the efficacy and safety of avatrombopag (Doptelet) for the treatment of paediatric patients with immune thrombocytopenia (ITP) of at least 6 months' duration. The study enrolled 75 subjects between 1 and <18 years and the primary endpoint, durable platelet response (6 out of 8 weekly platelet counts ≥50×10^9/L in the absence of rescue medication during weeks 5-12), was met in 28% of avatrombopag subjects in comparison with 0% of placebo subjects (p=0.0077, 95% CI 15.8-39.7).

The key secondary endpoint of two consecutive platelet counts ≥50×10^9/L was met in 81.5% of avatrombopag subjects in comparison with 0% of placebo subjects (p=<0.0001, 95% CI 71.1-91.8). A platelet response at Day 8 was observed in 56% of avatrombopag subjects and 0% of placebo subjects (p=<0.0001), while rescue therapy use occurred in 7% of avatrombopag subjects and 43% of placebo subjects (p=0.0008). The full study results, which will be presented at an upcoming medical conference, confirm that avatrombopag could be an effective and safe oral therapy for patients between 1 and <18 years with persistent and chronic ITP who have had an insufficient response to prior therapies.

"Based on these results, I believe that avatrombopag could provide a much-needed oral treatment option for adolescents and children with persistent and chronic ITP," said Rachael Grace, MD, MMSc, Paediatric haematologist and Director, Haematology Clinical Research at Dana-Farber/Boston Children's Cancer and Blood Disorders Center, and lead investigator of the AVA-PED-301 study. "In this paediatric population, the lack of any food restrictions or chronic immune suppression with avatrombopag treatment would be beneficial for patients living with ITP."

Immune thrombocytopenia (ITP) is an autoimmune disorder characterised by low numbers of platelets, leading to bruising and an increased risk of bleeding. It is estimated that up to 100 people per million live with ITP. In children, ITP occurs in 5 out of 100,000 children per year[1]. Currently no cure is available, and patients usually relapse after various treatments yet still require treatment to reduce the risk of clinically significant bleeding.

"Considering the challenges in treatment administration, coupled with variable and transient responses, frequent relapses, and associated toxicities from existing therapies, an unmet medical need currently exists in managing ITP among children and adolescents," said Lydia Abad-Franch, MD, MBA, Head of Research, Development, and Medical Affairs, and Chief Medical Officer at Sobi. "In line with Sobi's mission to transform the lives of people with rare and debilitating diseases, we will continue to work towards enhancing care and addressing the distinctive challenges faced by younger ITP patients and their caregivers."

References

1. Fogarty PF, Segal JB. The epidemiology of immune thrombocytopenic purpura. Curr Opin Hematol. 2007;14(5):515-519. doi:10.1097/MOH.0b013e3282ab98c7

About AVA-PED-301

AVA-PED-301 was a global, randomised, phase 3 study evaluating the efficacy, safety, and pharmacokinetics of Doptelet (avatrombopag) in the treatment of paediatric subjects with ITP. Eligible paediatric subjects with platelet counts <30×10^9/L entered a 12-week double-blind phase followed by an open-label extension phase up to 2 years in duration. The study enrolled a total of 75 paediatric subjects (aged ≥1 to <18 years of age) in 9 countries. Subjects were randomised to blinded therapy of either Doptelet or placebo in a 3:1 ratio. Enrolment into the study was 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 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. In June 2019, Doptelet was approved by the FDA for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment and in 2021, Doptelet was approved by EMA for the treatment of primary chronic ITP in adult patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Chronic ITP is a rare autoimmune bleeding disorder characterised by 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.

Sobi®

Sobi® is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology, and specialty care, Sobi has approximately 1,800 employees across Europe, North America, the Middle East, Asia, and Australia. In 2023, revenue amounted to SEK 22.1 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com and LinkedIn.

Contacts

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