

PRESS RELEASE

Stockholm, Sweden, 9 December 2019

Additional analyses from phase 3 study with Doptelet® (avatrombopag) for the treatment of chronic immune thrombocytopenia provides evidence of long-term response rates

Dova Pharmaceuticals, a wholly owned subsidiary of Swedish Orphan Biovitrum AB (publ) (Sobi™), presented additional analyses from the phase 3 study – core and extension phase - with avatrombopag (AVA) for the treatment of immune thrombocytopenia (ITP), at the 61st Annual Meeting of the American Society of Hematology (ASH) taking place in Orlando, 7-10 December.

Doptelet® (avatrombopag) is a novel, oral thrombopoietin receptor agonist (TPO-RA) approved by the FDA for the treatment of chronic immune thrombocytopenia (ITP) in adult patients who have had insufficient response to previous treatment. It is also approved for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a procedure. Avatrombopag is different than other orally available TPO-RAs in that it does not have a boxed warning for hepatotoxicity, is administered with food, and does not have any specific dietary restrictions. Further, it does not interact with polyvalent cations (calcium, magnesium, iron, selenium, zinc, etc.) in foods, mineral supplements, or antacids that could reduce systemic exposure.

Platelet response rates were similar in the core study and extension phase. Durable responders maintained a high rate of platelet response and complete platelet response in the extension phase, providing evidence that AVA-responding patients will likely continue to respond for extended treatment periods. Additionally, consistent efficacy was noted, as patients in the placebo group who rolled over to active drug demonstrated similar efficacy as AVA-treated patients. AVA-treated patients had substantially higher platelet response and complete platelet response rates than those treated with placebo.

“These results confirm the long-term response rates and efficacy of treatment with avatrombopag for the treatment of ITP”, says Kavita Aggarwal, Vice President Medical Affairs at Dova Pharmaceuticals.

Method and results from the study

A randomised, controlled, double-blind phase 3 study including 32 ITP patients treated with AVA and 17 treated with placebo for 6 months was previously publishedⁱ, demonstrating the superiority of AVA to placebo in median cumulative weeks achieving platelet counts (PC) $\geq 50,000/\mu\text{L}$ (12.4 vs. 0.0 weeks, $p < 0.0001$), and a rapid onset of action (65.6 per cent achieved PC $\geq 50,000/\mu\text{L}$ on day 8 for AVA vs. 0 per cent for placebo ($p < 0.0001$). In addition, AVA had a favourable safety profile with headache, fatigue, petechiae, contusion, and upper respiratory tract infection reported as the most common adverse events. Long-term AVA response rates in ITP have not previously been published.

After the phase 3 core study, patients had the option to continue to receive AVA in an open-label extension phase. Patients were eligible for the extension phase if they had completed the 6-month

core study or discontinued treatment due to lack of efficacy in the core study. A subset of “durable responders” who achieved a platelet response for 6 of the final 8 weeks of the core study (34.4 per cent of AVA patients qualified as durable responders), was also analysed separately to assess for maintenance of this high response rate in the extension phase.

The mean duration of study participation was 23 weeks in the core study and 44 (range, 8-76) weeks overall (core plus extension). In the core study, a platelet response ($PC \geq 50,000/\mu L$) was achieved at 48.6 per cent of patient visits for AVA, compared with <0.01 per cent for PBO. In the open-label extension phase for those patients continuing AVA treatment, a platelet response was achieved at 44.2 per cent of visits and in 41.4 per cent for PBO patients who rolled over to AVA in the extension phase. In durable responders from the core study (as defined above), a platelet response was achieved at 96.1 per cent of the extension phase visits.

About Doptelet® (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 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 1,300 people across Europe, North America, the Middle East, Russia and North Africa. In 2018, Sobi’s revenues amounted to SEK 9.1 billion. Sobi’s share (STO:SOBI) are listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

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ⁱ Jurczak et al., 2018