PRESS RELEASE

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FDA grants avatrombopag Orphan Drug Designation for the treatment of Chemotherapy-Induced Thrombocytopenia

Dova Pharmaceuticals, Inc., a wholly owned subsidiary of Swedish Orphan Biovitrum AB (publ) (Sobi™) has been granted Orphan Drug Designation[1] (ODD) by the US Food and Drug Administration (FDA) for avatrombopag for the potential treament of Chemotherapy-Induced Thrombocytopenia (CIT). Enrollment remains ongoing for the phase 3 clinical study for the treatment of patients with CIT.

CIT, a potentially serious complication of chemotherapy, results in low platelet levels and can lead to chemotherapy dose reductions, chemotherapy dose delays, or changes to chemotherapy regimens. For cancer patients receiving chemotherapy with curative intent, alterations in their chemotherapy regimen due to low platelets may compromise their long-term outcomes. Approximately 10 percent of US cancer patients per year experience CIT[2] which may require chemotherapy regimen modifications. Currently there are no approved treatments available for CIT in the US or EU.

“We are very pleased with the orphan drug designation from FDA for avatrombopag within CIT. Chemotherapy-induced low platelet counts are a crucial impediment for patients to be able to adhere to their chemotherapy regimen. Avatrombopag is currently being studied in a phase 3 study for this severe medical condition,” said Milan Zdravkovic, Head of Research and Development and Chief Medical Officer at Sobi.

Avatrombopag is approved as Doptelet® in both the US and the EU for the treatment of thrombocytopenia in adult patients with chronic liver disease (CLD) who are scheduled to undergo a procedure, and in the US for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment.

About Orphan Drug Designation

The Orphan Drug Designation programme provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the US, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Orphan designation qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act, such as a 50 percent tax credit on the cost of clinical trials undertaken in the US and a seven-year period of marketing exclusivity following market approval.[i]

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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 by FDA for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. Chronic ITP is a rare autoimmune bleeding disorder characterised by low number of platelets, affecting approximately 60,000 adults in the United States. Avatrombopag is not approved for chemotherapy-induced thrombocytopenia (CIT), a phase 3 study is currently ongoing.

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) is listed on Nasdaq Stockholm. You can find more information about Sobi at sobi.com.

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[i] FDA: Developing Products for Rare Diseases & Conditions.
https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm
[2] Weycker D et al. Risk and consequences of chemotherapy-induced thrombocytopenia in US clinical practice. BMC Cancer. 2019;19(1):151.