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Public Summary SwissPAR dated 23 March 2022

Doptelet® (active substance: avatrombopag)

First authorisation in Switzerland: 23 November 2021

Medicinal product for the treatment of severe thrombocytopenia and chronic immune thrombocytopenia in adults

About the medicinal product

Doptelet contains the active substance avatrombopag and is used for the treatment of severe thrombocytopenia in adults with chronic liver disease who are scheduled to undergo a surgical procedure (operation). Doptelet is also used for the treatment of adults with chronic immune thrombocytopenia (ITP) who have failed to respond adequately to at least one previous treatment.

Thrombocytopenia (TCP) is a blood disorder characterised by a reduced thrombocyte (platelet) count. TCP occurs in patients with chronic liver disease. As a result of their reduced platelet count, such patients are at increased risk of bleeding but need numerous surgical procedures.

Chronic immune thrombocytopenia (ITP) is an acquired thrombocytopenia caused by a reaction of the body's own defence system against platelets. This autoimmune response can also affect the megakaryocytes (precursor cells of platelets), leading to reduced platelet production. Depending on its duration, ITP is classified as acute (newly diagnosed; 0-3 months), persistent (3-12 months) or chronic (≥ 12 months).

Swissmedic evaluated the authorisation application for Doptelet for the treatment of severe thrombocytopenia in adults with chronic liver disease on the basis of the assessments of the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). To this end, Swissmedic referred to the publicly accessible assessment reports (www.ema.europa.eu and www.fda.gov).

In evaluating Doptelet, Swissmedic focused on the data on the treatment of chronic immune thrombocytopenia. Accordingly, the data and explanations in the SwissPAR and the resulting Public Summary SwissPAR primarily describe these results and conclusions.

Mode of action

Avatrombopag is a "thrombopoietin (TPO) receptor agonist[1]", which stimulates cell division, cell growth and the differentiation of megakaryocytes from bone marrow precursor cells, thereby increasing the platelet count.

Use

Doptelet is a prescription-only medicine and is authorised as a tablet containing 20 mg of the active substance avatrombopag.

Doptelet should always be taken at the same time of day together with a meal. The recommended daily dose of Doptelet is based on the platelet count in the blood.

A rise in the platelet count has been observed between 3 and 5 days after the start of treatment. The highest count was observed after 10 to 13 days. After the treatment, the platelet counts gradually decline again, returning to levels close to the original count.

Efficacy

The efficacy of Doptelet in the treatment of chronic immune thrombocytopenia was investigated in study 302 with a total of 49 participants.

In order to confirm the efficacy of Doptelet, some of the patients were treated for 6 months with Doptelet and some with a placebo (dummy drug).

The efficacy of Doptelet was demonstrated by the number of weeks in which the platelet counts were sufficiently high. In the patients treated with Doptelet, this number was higher than in the patients treated with placebo.

Precautions, undesirable effects & risks

Doptelet must not be used in those who are hypersensitive to the active substance or any of the excipients.
The most common side effects in all patients treated with Doptelet were headache and fatigue. Thromboembolic[2] events were recorded in 7% of the patients with chronic immune thrombocytopenia. If corresponding symptoms occur (e.g. feeling of heaviness, tingling, swelling), the doctor must be informed without delay.

All precautions, risks and other possible undesirable effects are listed in the Information for healthcare professionals.

Why the medicinal product has been authorised

The pivotal clinical trial confirmed the efficacy of Doptelet in adults with chronic ITP. Doptelet increased the platelet count quickly and sustainably.

[1] Receptor agonist: Receptors are very specific docking sites on a cell. Receptors exist for numerous substances. As soon as a specific substance (agonist) binds to its receptor, a reaction in the cell is triggered.

[2] Thromboembolism: A thromboembolic event is caused by a blood clot (thrombus) that blocks a site in the bloodstream or that is transported elsewhere in the bloodstream (embolus) and blocks another vessel (embolism).

Based on all the available data, the benefits of Doptelet outweigh the risks. Swissmedic has therefore authorised the medicinal product Doptelet for the treatment of adults with severe thrombocytopenia who are scheduled to undergo an invasive procedure and for the treatment of adults with chronic immune thrombocytopenia who have failed to respond adequately to at least one previous treatment.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Doptelet®

Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

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