

Real-world data show Novartis drug Promacta® improves outcomes for ITP patients compared to other second-line therapies

Dec 02, 2018

- - Promacta (eltrombopag) showed lower rate of bleeding-related episodes and similar rate of thrombotic events vs. romiplostim, rituximab and splenectomy, in a retrospective analysis of US electronic health records
- - Patients who received splenectomy, as second-line regimen, showed highest platelet counts and most frequent thrombotic event rates among groups receiving other therapies
- - Immune thrombocytopenia (ITP) is a rare blood disorder where there is an increased risk of bleeding due to a low number of platelets

EAST HANOVER, N.J., Dec. 2, 2018 /PRNewswire/ -- Novartis announced results of a retrospective, real-world evidence study in patients with immune thrombocytopenia (ITP) treated with Promacta® (eltrombopag), compared to other second-line therapies. The data demonstrated that patients experienced better clinical outcomes with Promacta, in terms of fewer bleeding episodes. The data were presented during the 60th Annual Meeting of the American Society of Hematology (ASH) in San Diego.

"Despite advances in treating immune thrombocytopenia, many patients remain at risk for bleeding episodes," said Samit Hirawat, MD, Head, Novartis Oncology Global Drug Development. "With these kind of real-world data, we can reimagine care by more clearly understanding the outcomes of a range of treatments and, in turn, helping healthcare providers better navigate available options with their patients."

Electronic health records (EHR) data from January 1, 2009 to September 30, 2016 from the Optum® EHR database were used to evaluate the effect of second-line agents for ITP. Identified patients had the following characteristics: 18 years or older, evidence of previous treatment with steroids or immune globulin products, and activity in the database for at least 6 months prior to and 12 months post initiation of a second-line agent. Treatment outcomes evaluated included platelet counts, bleeding related episodes (BREs), and thrombotic events (TEs) over the 12-month period following starting a second-line therapy.

Of the 2,526 adults that met the inclusion criteria, 110 (4.4%) received eltrombopag, 189 (7.5%) romiplostim, 1,488 (58.9%) rituximab, and 260 (10.3%) splenectomy, with the remaining 479 (18.9%) receiving a mix of other second-line agents. Compared to baseline, platelet counts increased in all treatment cohorts. The proportion of patients who experienced BREs ranged from 25.5% (eltrombopag) to 36.5% (romiplostim), while TEs were observed in all treatment cohorts ranging from 11.6% (eltrombopag) to 15.7% (splenectomy). An additional analysis demonstrated that patients with ITP who had a splenectomy as second-line treatment had the highest mean platelet counts during the first 12 months post treatment initiation, but were at greatest risk for TEs (15.7%) (e.g., stroke, transient ischemic attack, myocardial infarction, deep vein thrombosis, and pulmonary embolism) compared to 11.6% (eltrombopag), 12.7% (romiplostim), and 13.9% (rituximab).

"These real-world data can help doctors as they weigh options for second-line therapy with their patients." Adam Cuker, MD, Assistant Professor of Medicine at the University of Pennsylvania. "They may also help explain the long-term trend toward deferring splenectomies until after other lines of treatment have been tried."

Immune thrombocytopenia is a rare and potentially serious blood disorder where there is an increased risk of bleeding due to a low number of platelets. As a result, patients with ITP experience bruising, bleeding and, in rare cases, serious hemorrhage that can be fatal.¹ The goal of treatment in chronic/persistent ITP is to maintain a safe platelet count that reduces the risk of bleeding.¹

Promacta[®]/Revolade[®] (eltrombopag)

Eltrombopag, marketed as Promacta[®] in the US and Revolade[®] in countries outside the US, is approved in more than 90 countries worldwide for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenic purpura (ITP) who have had an inadequate response or are intolerant to other treatments. It is also approved for the treatment of patients with severe aplastic anemia (SAA) as first-line therapy in the US (patients 2 years and older) and Japan, and in many other countries for patients who are refractory to other treatments. In more than 40 countries, Promacta/Revolade is indicated for the treatment of thrombocytopenia in patients with chronic hepatitis C to allow them to initiate and maintain interferon-based therapy.

Promacta/Revolade is approved in the US and in the European Union for the treatment of thrombocytopenia in pediatric patients 1 year and older with chronic immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Promacta should only be used in patients with ITP whose degree of thrombocytopenia and clinical condition increase the risk for bleeding.

Important Safety Information

Promacta can cause serious side effects, including liver problems, abnormal liver function tests, high platelet counts and higher risk for blood clots, and new or worsened cataracts (a clouding of the lens in the eye).

Promacta is not for treatment of people with a precancerous condition called myelodysplastic syndromes (MDS). If you have MDS and receive Promacta, your MDS condition may worsen and become AML. If MDS worsens to become AML, you may die sooner from AML.

For patients who have chronic hepatitis C virus and take Promacta with interferon and ribavirin treatment, Promacta may increase the risk of liver problems. Patients should tell a healthcare provider right away if they have any of these signs and symptoms of liver problems including yellowing of the skin or the whites of the eyes (jaundice), unusual darkening of the urine, unusual tiredness, right upper stomach area pain, confusion, swelling of the stomach area (abdomen).

A healthcare provider will order blood tests to check the liver before starting Promacta and during Promacta treatment. In some cases, treatment with Promacta may need to be stopped due to changes in liver function tests.

The risk of getting a blood clot is increased if the platelet count is too high during treatment with Promacta. The risk of getting a blood clot may also be increased during treatment with Promacta if platelet counts are normal or low. Some forms of blood clots, such as clots that travel to the lungs or that cause heart attacks or strokes can cause severe problems or death. A healthcare provider will check blood platelet counts, and change the dose of Promacta or stop Promacta, if platelet counts get too high. Patients should tell a healthcare provider right away if they have signs and symptoms of a blood clot in the leg, such as swelling, pain, or tenderness in the leg.

People with chronic liver disease may be at risk for a type of blood clot in the stomach area. Patients should tell a healthcare provider right away if they have stomach area pain that may be a symptom of this type of blood clot.

New or worsened cataracts have happened in people taking Promacta. A healthcare provider will check the patient's eyes before and during treatment with Promacta. Patients should tell a healthcare provider about any

changes in eyesight while taking Promacta.

Patients should tell a healthcare provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Promacta may affect the way certain medicines work. Certain medicines may keep Promacta from working correctly. Patients should take Promacta at least 4 hours before or 4 hours after taking products such as antacids used to treat stomach ulcers or heartburn and multivitamins or products that contain iron, calcium, aluminum, magnesium, selenium, and zinc, which may be found in mineral supplements. Patients should ask a healthcare provider if they are not sure if the medicine is one that is listed above.

Patients should avoid situations and medications that may increase the risk of bleeding while taking Promacta.

The most common side effects of Promacta when used to treat chronic ITP in adults are: nausea; diarrhea; upper respiratory tract infection (symptoms may include runny nose, stuffy nose, and sneezing); vomiting; muscle aches; urinary tract infection (symptoms may include frequent or urgent need to urinate, low fever in some people, pain or burning with urination); pain or swelling (inflammation) in the throat or mouth (oropharyngeal pain and pharyngitis); abnormal liver function tests; back pain; flu-like symptoms (influenza), including fever, headache, tiredness, cough, sore throat, and body aches; skin tingling, itching, or burning; and rash.

The most common side effects of Promacta in children 1 year and older when used to treat chronic ITP are: upper respiratory tract infections (symptoms may include runny nose, stuffy nose, and sneezing); pain or swelling (inflammation) in the nose and throat (nasopharyngitis); cough; diarrhea; pyrexia; runny, stuffy nose (rhinitis); stomach (abdominal) pain; pain or swelling (inflammation) in the throat or mouth; toothache; abnormal liver function tests; rash; runny nose (rhinorrhea).

The most common side effects when Promacta is used in combination with other medicines to treat chronic HCV are: low red blood cell count (anemia); fever; tiredness; headache; nausea; diarrhea; decreased appetite; flu-like symptoms (influenza), including fever, headache, tiredness, cough, sore throat, and body aches; feeling weak; trouble sleeping; cough; itching; chills; muscle aches; hair loss; and swelling in the ankles, feet, and legs.

The most common side effects of Promacta when used to treat severe aplastic anemia (SAA) are: nausea, feeling tired, cough, diarrhea, headache, pain in arms, legs, hands or feet, shortness of breath, fever, dizziness, pain in nose or throat, abdominal pain, bruising, muscle spasms, abnormal liver function tests, joint pain, and runny nose. Laboratory tests may show abnormal changes to the cells in bone marrow.

The most common side effects of Promacta when used to treat adults and pediatric patients 2 years and older with SAA in combination with standard immunosuppressive therapy are: abnormal liver function tests, rash and skin discoloration including darkening of skin patches (hyperpigmentation).

Please see full [Prescribing Information](#), including Boxed WARNING and Medication Guide, for Promacta®.

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