Novartis drug Promacta® receives FDA Breakthrough Therapy designation for first-line use in severe aplastic anemia (SAA)

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- - Data supporting designation showed over half of treatment-naïve SAA patients achieved complete response with Promacta when given with standard immunosuppressive therapy, with overall response rate of 85%[1]
- - Promacta is the only TPO receptor agonist indicated for the treatment of patients with SAA, currently in the refractory setting
- - Regulatory filings of the first-line indication in US and EU expected in 2018

EAST HANOVER, N.J., Jan. 4, 2018 /PRNewswire/ -- Novartis today announced that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to Promacta® (eltrombopag) for use in combination with standard immunosuppressive therapy for the treatment of patients with severe aplastic anemia (SAA) as a first-line therapy. Promacta, which is marketed as Revolade® in most countries outside the US, is already approved as a second-line therapy in the refractory setting in SAA. Promacta is also approved for adults and children with chronic immune thrombocytopenia (ITP), for patients who are refractory to other treatments.

SAA is a rare blood disorder in which a patient's bone marrow fails to produce enough red blood cells, white blood cells and platelets[2]. As a result, people living with this serious disease may experience debilitating symptoms and complications, such as fatigue, trouble breathing, recurring infections and abnormal bruising or bleeding that can limit their daily activities[2]. Up to one-third of patients do not respond to current therapies or relapse, causing symptoms to return[3].

"Promacta is a promising medicine that, if approved for first-line use in severe aplastic anemia, may redefine the standard of care for patients with this rare and serious bone marrow condition," said Samit Hirawat, MD, Head, Novartis Oncology Global Drug Development. "We will continue to work closely with the FDA to make Promacta available to patients with SAA who are new to treatment as soon as possible."

Novartis' analysis of research conducted by the National Heart, Lung and Blood Institute (NHLBI) of the National Institutes of Health (NIH) showed that over half (52%) of patients with treatment-naïve SAA achieved complete response at six months when treated with Promacta at the initiation of and concurrently with standard immunosuppressive treatment. The overall response rate was 85%.

According to FDA guidelines, treatments that receive Breakthrough Therapy designation are those that treat a serious or life threatening disease or condition and demonstrate a substantial improvement over existing therapies on one or more clinically significant endpoints based on preliminary clinical evidence.

About Promacta® (eltrombopag)

Eltrombopag, marketed as Promacta® in the United States and Revolade® in countries outside the US, is approved in more than 100 countries worldwide for the treatment of thrombocytopenia in adult patients with

chronic immune (idiopathic) thrombocytopenic purpura (ITP) who have had an inadequate response or are intolerant to other treatments, approved in over 45 countries worldwide for the treatment of patients with severe aplastic anemia (SAA) who are refractory to other treatments, and also approved in more than 50 countries for the treatment of thrombocytopenia in patients with chronic hepatitis C to allow them to initiate and maintain interferon-based therapy. Eltrombopag 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 (idiopathic) thrombocytopenia (ITP) who have had an insufficient response to corticosteroids and immunoglobulins.

Important Safety Information for Promacta® (eltrombopag)
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 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.

Please see full Prescribing Information, including Boxed WARNING and Medication Guide, for Promacta.

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About Novartis
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List of links present in page

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