

Promacta[®] (eltrombopag) – Expanded indication

- On November 16, 2018, [Novartis announced](#) the FDA approval of [Promacta \(eltrombopag\)](#), in combination with standard immunosuppressive therapy for the first-line treatment of adult and pediatric patients 2 years and older with severe aplastic anemia (SAA).
 - Promacta was previously approved for the treatment of patients with SAA who have had an insufficient response to immunosuppressive therapy.
- Promacta is also approved for treatment of thrombocytopenia in patients with chronic immune thrombocytopenia and thrombocytopenia in patients with hepatitis C infection.
- SAA is a rare, life-threatening, acquired blood disorder in which a patient's bone marrow fails to produce enough red blood cells, white blood cells, and platelets. Individuals living with SAA may experience debilitating symptoms and complications, such as fatigue, trouble breathing, recurring infections, and abnormal bruising or bleeding that can limit their daily activities.
- The expanded indication for Promacta was based on data from a single-arm study in 92 patients with SAA who had not received prior immunosuppressive therapy. The efficacy of Promacta in combination with [horse antithymocyte globulin \(h-ATG\)](#) and [cyclosporine](#) was established on the basis of complete hematological response at 6 months. A key secondary endpoint was overall response rate, defined as the number of partial responses plus complete responses.
 - A complete response was achieved in 44% (95% CI: 33, 55) of patients.
 - The overall response rate was 79% (95% CI: 69, 87).
 - In the 34 patients between 2 to 16 years of age, 7 and 17 out of 25 patients achieved a complete and overall response, respectively, at 6 months.
- Promacta carries boxed warnings for a risk of hepatic decompensation in patients with chronic hepatitis C and a risk of hepatotoxicity.
- The recommended initial dose of Promacta for first-line treatment of SAA is 150 mg, 75 mg, and 2.5 mg/kg in patients 12 years and older, pediatric patients 6 to 11 years, and pediatric patients 2 to 5 years, respectively, orally once daily, for 6 months.
 - Promacta should be initiated concurrently with standard immunosuppressive therapy.
 - For patients with SAA of Asian ancestry or those with mild, moderate, or severe hepatic impairment (Child-Pugh Class A, B, C), the initial Promacta dose should be decreased by 50%.
 - Refer to the Promacta drug label for dosing information for all other indications.