

Specialty Pharmacy Program

Nplate[®] (romiplostim) and Promacta[®] (eltrombopag)

CLINICAL BACKGROUND

Immune (idiopathic) thrombocytopenic purpura (ITP) is an autoimmune disorder in which anti-platelet antibodies accelerate the destruction and decrease the production of platelets. It is characterized by reduced platelet count and mucocutaneous bleeding. The course and prognosis of ITP is mainly determined by the risk of spontaneous bleeding associated with low platelet counts. Therefore, the goal of treatment is to maintain a safe platelet count to prevent major bleeding while minimizing adverse effects. Treatment is indicated in patients with platelet counts less than $30 \times 10^9/L$ and in patients who have platelet counts less than $50 \times 10^9/L$ and have substantial mucous membrane bleeding or risk factors for bleeding.

While current treatments such as corticosteroids and intravenous immunoglobulins increase platelet counts primarily by reducing the extent of platelet destruction, recognition of decreased platelet production in patients with ITP has led to the use of treatments that enhance thrombopoiesis. Nplate and Promacta, recombinant thrombopoietin receptor agonists, are indicated for the treatment of thrombocytopenia in patients with chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Nplate is administered as a weekly subcutaneous injection and Promacta is administered orally once daily.

Nplate and Promacta produce dose-dependent increases in platelet counts. The doses of Nplate and Promacta are adjusted according to platelet counts to reduce the risk for bleeding. Nplate and Promacta are available only through restricted distribution programs. Promacta may cause hepatotoxicity and liver function tests should be monitored routinely during treatment.

APPROVAL DURATION

Initial authorization: 6 months

Re-authorization: 3-12 months

APPROVAL CRITERIA

FDA-Approved Indication(s)

1. Treatment of thrombocytopenia in patients with chronic immune (idiopathic) thrombocytopenic purpura (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.