**OVERVIEW:** Promacta, a thrombopoietin receptor agonist, has three indications. Promacta is indicated for the treatment of thrombocytopenia in patients with chronic immune (idiopathic) thrombocytopenic purpura (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Promacta is indicated for the treatment of patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy. Promacta is also indicated for the treatment of thrombocytopenia in patients with chronic hepatitis C (CHC) to allow initiation and maintenance of interferon-based therapy. Promacta should only be used in those with ITP whose degree of thrombocytopenia and clinical condition increase the bleeding risk. Promacta should be used only in patients with CHC whose degree of thrombocytopenia prevents the initiation of interferon-based therapy or limits the ability to maintain interferon-based therapy. The safety and efficacy of Promacta have not been established in combination with direct-acting antiviral agents indicated for the treatment of CHC genotype 1 infection. Promacta has a boxed warning regarding the risk for hepatic decompensation in patients with CHC.

**POLICY STATEMENT**
Prior authorization is recommended for prescription benefit coverage of Promacta. All approvals are provided for 1 year in duration unless otherwise noted below. In cases where the approval is authorized in months, 1 month is equal to 30 days.

**Automation:** None.

**RECOMMENDED AUTHORIZATION CRITERIA**
Coverage of Promacta is recommended in those who meet the following criteria:

**Food and Drug Administration (FDA)-Approved Indications**

1. **Treatment of Thrombocytopenia in Patients with Chronic Immune (Idiopathic) Thrombocytopenic Purpura (ITP).** Approve Promacta if the patient meets the following criteria (a, b, c, and d):

   a. The agent is prescribed by, or after consultation with, a hematologist; AND
   b. The patient is ≥ 1 year of age; AND
   c. Not using Promacta in combination with Nplate; AND
   d. The patient meets ONE of the following conditions (i, ii, iii, or iv):
      i. The patient is not a candidate for corticosteroids, IVIG or a splenectomy; OR
      ii. The patient has tried corticosteroids; OR
      iii. The patient has tried IVIG; OR
      iv. The patient has undergone splenectomy.

Promacta is indicated for the treatment of thrombocytopenia in adult and pediatric patients aged ≥ 1 year with chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Most pivotal trials with Promacta involved patients who had tried at least one primary
ITP therapy (e.g., corticosteroids, immunoglobulins) or had undergone splenectomy. Evidence-based practice guidelines for immune thrombocytopenia from ASH (published in 2011), recommends corticosteroids or IVIG as first-line treatment for adults; splenectomy is recommended for patients who have failed corticosteroid therapy. Thrombopoietin receptor agonists are recommended for adults at risk of bleeding who relapse following splenectomy or who have a contraindication to splenectomy and who have failed at least one other therapy.

2. **Treatment of Thrombocytopenia in Patients with Chronic Hepatitis C.** Approve Promacta if the patient meets the following criteria (a, b, c, and d):

   a. Promacta is prescribed by, or after consultation with, either a gastroenterologist, a hepatologist, or a physician that specializes in infectious disease; AND
   
   b. The patient has low platelet counts at baseline (pretreatment) [e.g., < 75,000 mm$^3$]; AND
   
   c. The patient will be receiving interferon-based therapy for chronic hepatitis C (e.g., pegylated interferon [Pegasys® {peginterferon alfa-2a injection}, PegIntron® {peginterferon alfa-2b injection}, or Intron A® {interferon alfa-2b}]); AND
   

Promacta is indicated for the treatment of thrombocytopenia in patients with CHC to allow the initiation and maintenance of interferon-based therapy. It should only be used in patients with CHC whose degree of thrombocytopenia prevents the initiation of interferon-based therapy or limits the ability to maintain interferon-based therapy. Patients in the trials were adults with CHC who were receiving either PegIntron or Pegasys, along with ribavirin, and platelet counts were < 75 x 10$^9$/L. Use of Promacta allowed approximately 95% of patients to initiate CHC therapy and a statistically significantly greater proportion of patients given Promacta achieved SVR.

3. **Aplastic Anemia.** Approve Promacta if the patient meets the following criteria (a, b, c, d, and e):

   a. The patient has low platelet counts at baseline (pretreatment) [e.g., < 30,000 mm$^3$]; AND
   
   b. Promacta is prescribed by, or after consultation with, a hematologist; AND
   
   c. That patient had tried one immunosuppressant therapy (e.g., cyclosporine, mycophenolate mofetil, sirolimus, Atgam® [lymphocyte immune globulin, anti-thymocyte globulin [equine] sterile solution for intravenous use only]); AND
   
   d. The patient is ≥ 18 years of age; AND
   
   e. Not using Promacta in combination with Nplate.

Promacta is indicated for the treatment of patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy. The data that led to approval of Promacta involved patients who had an insufficient response to at least one prior immunosuppressive therapy and with low initial platelet counts (≤ 30 x 10$^9$/L). Other immunosuppressive therapies used in aplastic anemia include cyclosporine, Atagam, mycophenolate mofetil, and sirolimus.

4. **Patient Has Been Started on Promacta.** Approve for an indication or condition addressed as an approval in the Recommended Authorization Criteria section (FDA-Approved Indications) if the patient meets the following criteria (a and b):

   a. Patient continues to respond to therapy with this drug (e.g. platelet count has increased); AND

Other Uses with Supportive Evidence

5. Thrombocytopenia in Myelodysplastic Syndrome (MDS). Approve Promacta for 12 months if the patient meets the following criteria (A, B, C and D):

A) The agent is prescribed by, or in consultation with, a hematologist or an oncologist; AND
B) The patient has low- to intermediate-risk MDS; AND
C) According to the prescribing physician the patient has clinically significant thrombocytopenia (e.g., low platelet counts [< 30,000 mm\(^3\) [pretreatment]]; is platelet transfusion-dependent; active bleeding, and/or a history of bleeding at low platelet counts); AND
D) Patient is not using Promacta in combination with Nplate

Conditions Not Recommended for Approval
Promacta has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. Rationale for non-coverage for these specific conditions is provided below. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval.)

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES


OTHER REFERENCES UTILIZED