Reference number(s)
1928-A

SPECIALTY GUIDELINE MANAGEMENT

PROMACTA (eltrombopag)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

A. FDA-Approved Indications
1. Treatment of thrombocytopenia in adult and pediatric patients 1 year and older with persistent or chronic immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy
2. Treatment of thrombocytopenia in patients with chronic hepatitis C to allow the initiation and maintenance of interferon-based therapy
3. First-line treatment of severe aplastic anemia in adult and pediatric patients 2 years and older in combination with standard immunosuppressive therapy
4. Treatment of patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy

B. Compendial Uses
1. MYH9-related disease with thrombocytopenia
2. Myelodysplastic syndromes, for lower risk disease in patients with severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents, immunosuppressive therapy, or clinical trial.
3. Myelodysplastic syndromes, in combination with equine anti-thymocyte globulin with or without cyclosporine, for treatment of lower risk disease in select patients (generally ≤60 years old and with ≤5% marrow blasts, or those with hypocellular marrows, PNH clone positivity, or STAT-3 mutant cytotoxic T-cell clones) with clinically relevant thrombocytopenia or neutropenia.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Chronic or persistent immune thrombocytopenia: pretreatment and current platelet counts
B. Aplastic anemia continuation of therapy: current platelet counts

III. EXCLUSIONS

Coverage will not be provided for members with the following exclusion: concomitant use of Promacta with other thrombopoietin receptor agonists (e.g., Nplate, Doptelet, Mulpleta) or with spleen tyrosine kinase inhibitors (e.g., Tavalisse)

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IV. PRESCRIBER SPECIALTIES

A. For diagnosis of persistent or chronic thrombocytopenia, severe aplastic anemia, MYH9-related disease with thrombocytopenia and myelodysplastic syndromes, this medication must be prescribed by or in consultation with a hematologist or oncologist.
B. For diagnosis of hepatitis C, this medication must be prescribed by or in consultation with a hepatologist, gastroenterologist, or infectious disease specialist.

V. CRITERIA FOR INITIAL APPROVAL

A. Chronic or persistent immune thrombocytopenia (ITP)
Authorization of 6 months may be granted for treatment of chronic or persistent ITP when both of the following criteria are met:
1. Inadequate response or intolerance to prior therapy with corticosteroids, immunoglobulins, or splenectomy
2. Untransfused platelet count at any point prior to the initiation of the requested medication is less than $30x10^9$/L OR $30x10^9$/L to $50x10^9$/L with symptomatic bleeding (e.g., significant mucous membrane bleeding, gastrointestinal bleeding or trauma) or risk factors for bleeding (see Section VII).

B. Thrombocytopenia associated with chronic hepatitis C
Authorization of 6 months may be granted to members who are prescribed Promacta for the initiation and maintenance of interferon-based therapy for the treatment of thrombocytopenia associated with chronic hepatitis C.

C. Aplastic anemia
1. Authorization of 6 months may be granted for first-line treatment of severe aplastic anemia when Promacta will be used in combination with standard immunosuppressive therapy (e.g., horse antithymocyte globulin (h-ATG) and cyclosporine).
2. Authorization of 6 months may be granted for treatment of aplastic anemia which had an insufficient response to immunosuppressive therapy.

D. MYH9-related disease with thrombocytopenia
Authorization of 12 months may be granted to members with thrombocytopenia associated with MYH9-related disease.

E. Myelodysplastic Syndromes
1. Authorization of 12 months may be granted for treatment of myelodysplastic syndromes with severe or refractory thrombocytopenia when both of the following criteria are met:
   i. Member has lower risk disease defined as Revised International Prognostic Scoring System (IPSS-R) (Very Low, Low, Intermediate), International Prognostic Scoring System (IPSS) (Low/Intermediate-1), WHO classification-based Prognostic Scoring System (WPSS) (Very Low, Low, Intermediate).
   ii. Member has severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents (such as azacitidine and decitabine), immunosuppressive therapy, or clinical trial.
2. Authorization of 12 months may be granted for treatment of myelodysplastic syndromes when all of the following criteria are met:
   i. Member has lower risk disease defined as Revised International Prognostic Scoring System (IPSS-R) (Very Low, Low, Intermediate), International Prognostic Scoring System (IPSS) (Low/Intermediate-1), WHO classification-based Prognostic Scoring System (WPSS) (Very Low, Low, Intermediate).
   ii. Member has clinically relevant thrombocytopenia or neutropenia.

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iii. Promacta will be used in combination with equine anti-thymocyte globulin.

VI. CONTINUATION OF THERAPY

A. Chronic or persistent ITP

1. Authorization of 3 months may be granted to members with current platelet count less than $50x10^9$/L for whom the platelet count is not sufficient to prevent clinically important bleeding and who have not received a maximal Promacta dose for at least 4 weeks.
2. Authorization of 12 months may be granted to members with current platelet count less than $50x10^9$/L for whom the current platelet count is sufficient to prevent clinically important bleeding.
3. Authorization of 12 months may be granted to members with current platelet count of $50x10^9$/L to $200x10^9$/L.
4. Authorization of 12 months may be granted to members with current platelet count greater than $200x10^9$/L to less than or equal to $400x10^9$/L for whom Promacta dosing will be adjusted to achieve a platelet count sufficient to avoid clinically important bleeding.

B. Thrombocytopenia associated with chronic hepatitis C

Authorization of 6 months may be granted to members who are continuing to receive interferon-based therapy.

C. Aplastic anemia

1. Authorization of up to 16 weeks total may be granted to members with current platelet count less than $50x10^9$/L who have not received appropriately titrated therapy with Promacta for at least 16 weeks.
2. Authorization of up to 16 weeks total may be granted to members with current platelet count less than $50x10^9$/L who are transfusion-independent.
3. Authorization of 12 months may be granted to members with current platelet count of $50x10^9$/L to $200x10^9$/L.
4. Authorization of 12 months may be granted to members with current platelet count greater than 200 $x10^9$/L to less than or equal to $400x10^9$/L for whom Promacta dosing will be adjusted to achieve and maintain an appropriate target platelet count.

D. MYH9-related disease with thrombocytopenia

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

E. Myelodysplastic Syndromes

Authorization of 12 months may be granted for continued treatment of myelodysplastic syndromes in members who experience benefit from therapy (e.g., increased platelet counts, decreased bleeding events, reduced need for platelet transfusions).

VII. APPENDIX

<u>Examples of risk factors for bleeding (not all inclusive)</u>

- Undergoing a medical or dental procedure where blood loss is anticipated
- Comorbidity (e.g., peptic ulcer disease, hypertension)
- Mandated anticoagulation therapy
- Profession (e.g., construction worker) or lifestyle (e.g., plays contact sports) that predisposes member to trauma

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