

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 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 or immunosuppressive therapy
3. Severe thrombocytopenia post cancer chemotherapy

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 primary immune thrombocytopenia: pretreatment and current platelet counts
- B. Aplastic anemia continuation of therapy: current platelet counts
- C. Severe thrombocytopenia post cancer chemotherapy: pretreatment and 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)

IV. CRITERIA FOR INITIAL APPROVAL

A. Chronic or persistent primary 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 time of diagnosis is less than $30 \times 10^9/L$ OR $30 \times 10^9/L$ to $50 \times 10^9/L$ with symptomatic bleeding (e.g., significant mucous membrane bleeding, gastrointestinal bleeding or trauma) or risk factors for bleeding (see Section VI).

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 has been previously treated with 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

Authorization of 12 months may be granted for treatment of myelodysplastic syndromes when both of the following criteria are met:

1. 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).
2. Member has severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents (such as azacitidine and decitabine) or immunosuppressive therapy.

F. Severe thrombocytopenia post cancer chemotherapy

Authorization of 6 months may be granted for treatment of severe thrombocytopenia post cancer chemotherapy when the platelet count is less than $50 \times 10^9/L$.

V. CONTINUATION OF THERAPY

A. Chronic or persistent ITP

1. Authorization of 3 months may be granted to members with current platelet count less than $50 \times 10^9/L$ 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 $50 \times 10^9/L$ 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 $50 \times 10^9/L$ to $200 \times 10^9/L$.

4. Authorization of 12 months may be granted to members with current platelet count greater than $200 \times 10^9/L$ to less than or equal to $400 \times 10^9/L$ 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 $50 \times 10^9/L$ 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 $50 \times 10^9/L$ who are transfusion-independent.
3. Authorization of 12 months may be granted to members with current platelet count of $50 \times 10^9/L$ to $200 \times 10^9/L$.
4. Authorization of 12 months may be granted to members with current platelet count greater than $200 \times 10^9/L$ to less than or equal to $400 \times 10^9/L$ 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).

F. Severe thrombocytopenia post cancer chemotherapy

Authorization of 6 months may be granted for continued treatment of severe thrombocytopenia post cancer chemotherapy in members who experience benefit from therapy (e.g., increased platelet counts, decreased bleeding events, reduced need for platelet transfusions) and the platelet count remains less than $100 \times 10^9/L$.

VI. APPENDIX

Examples of risk factors for bleeding (not all inclusive)

- 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 patient to trauma

VII. REFERENCES

1. Promacta [package insert]. Research Triangle Park, NC: GlaxoSmithKline; April 2019.
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8. Olnes MJ, Scheinberg P, Calvo KR, et al. Eltrombopag and improved hematopoiesis in refractory aplastic anemia. *N Engl J Med*. 2012;367(1):11-19.
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10. NCCN hematopoietic growth factors. Short-term recommendations specific to issues with COVID-19 (SARS-CoV-2). National Comprehensive Cancer Network, Inc. Available at: https://www.nccn.org/covid-19/pdf/HGF_COVID-19.pdf. Accessed April 16, 2020.