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 chronic immune (idiopathic) 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 Use
1. MYH9-related disease with thrombocytopenia

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR INITIAL APPROVAL

A. Chronic or persistent primary immune thrombocytopenia (ITP)
Authorization of 6 months may be granted to members with chronic or persistent ITP who meet all of the following criteria:
1. Inadequate response or intolerance to documented prior therapy with corticosteroids, immunoglobulins, or splenectomy
2. Untransfused platelet count at time of diagnosis 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 IV).

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 to members for the treatment of first-line 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 to members for the 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.

III. CONTINUATION OF THERAPY

A. Chronic or persistent ITP
1. Authorization of 12 months may be granted to members with current platelet count less than or equal to $200x10^9$/L.
2. Authorization of 12 months may be granted to members with current platelet count greater than 200 $x10^9$/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 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.

IV. 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

V. REFERENCES
1. Promacta [package insert]. Research Triangle Park, NC: GlaxoSmithKline; November 2018.
2. Pecci A, Gresele P, Klersy C, et al. Eltrombopag for the treatment of the inherited thrombocytopenia deriving from MYH9 mutations. *Blood.* 2010;116(26):5832-7.

3. Neunert C, Lim W, Crowther M, et al. The American Society of Hematology 2011 evidence-based practice guideline for immune thrombocytopenia. *Blood.* 2011;117(16):4190-4207.
4. Provan D, Stasi R, Newland AC, et al. International consensus report on the investigation and management of primary immune thrombocytopenia. *Blood.* 2010;115(2):168-186.
5. Rodeghiero F, Stasi R, Gernsheimer T, et al. Standardization of terminology, definitions and outcome criteria in immune thrombocytopenic purpura of adults and children: report from an international working group. *Blood*. 2009;113(11):2386-2393.
6. 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.
7. Townsley DM, Scheinberg P, Winkler T, et al. Eltrombopag added to standard immunosuppression for aplastic anemia. N Engl J Med 2017;376:1540-1550.