Reference number(s)
1928-A

Specialty Guideline Management
Promacta-Alvaiz

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Promacta	eltrombopag olamine
Alvaiz	eltrombopag choline

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.

FDA-approved Indications

Promacta is indicated for:

- 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.
- Treatment of thrombocytopenia in patients with chronic hepatitis C to allow the initiation and maintenance of interferon-based therapy.
- First-line treatment of severe aplastic anemia in adult and pediatric patients 2 years and older in combination with standard immunosuppressive therapy.
- Treatment of patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy.

Alvaiz is indicated for:

- Treatment of thrombocytopenia in adult and pediatric patients 6 years and older with persistent or chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
- Treatment of thrombocytopenia in adult patients with chronic hepatitis C to allow the initiation and maintenance of interferon-based therapy.
- Treatment of adult patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy.

Compendial Uses

- MYH9-related disease with thrombocytopenia (Promacta only)
- Myelodysplastic syndromes (MDS) (Promacta only)
- Thrombocytopenia post-hematopoietic cell transplant

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

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Persistent or chronic immune thrombocytopenia (ITP):
  - For initial requests: pretreatment platelet count
  - For continuation requests: current platelet count
- Aplastic anemia continuation of therapy: current platelet count

Exclusions

Coverage will not be provided when the requested drug will be used concomitantly with other thrombopoietin receptor agonists (e.g., Nplate, Doptelet, Mulpleta) or with spleen tyrosine kinase inhibitors (e.g., Tavalisse).

Prescriber Specialties

This medication must be prescribed by or in consultation with either of the following:

- Persistent or chronic immune thrombocytopenia (ITP), aplastic anemia, MYH9-related disease with thrombocytopenia, myelodysplastic syndromes, and thrombocytopenia post-hematopoietic cell transplant: hematologist or oncologist

- Thrombocytopenia with hepatitis C: hematologist or a prescriber specializing in infectious disease, gastroenterology, hepatology, or transplant

Coverage Criteria

Persistent or chronic immune thrombocytopenia (ITP)

Authorization of 6 months may be granted for treatment of persistent or chronic ITP when both of the following criteria are met:

- Member has had an inadequate response or intolerance to prior therapy with corticosteroids, immunoglobulins, or splenectomy.
- Member has an untransfused platelet count at any point prior to the initiation of the requested medication of either of the following:
  - Less than $30x10^9$/L
  - $30x10^9$/L to $50x10^9$/L with symptomatic bleeding (e.g., significant mucous membrane bleeding, gastrointestinal bleeding or trauma) or risk factors for bleeding (see Appendix)

Thrombocytopenia associated with chronic hepatitis C

Authorization of 12 months may be granted to members who are prescribed the requested drug for the initiation and maintenance of interferon-based therapy for the treatment of thrombocytopenia associated with chronic hepatitis C.

Severe aplastic anemia

Promacta

- Authorization of 6 months may be granted for first-line treatment of severe aplastic anemia when the requested drug will be used in combination with standard immunosuppressive therapy (e.g., horse antithymocyte globulin [h-ATG] and cyclosporine).
- Authorization of 6 months may be granted for treatment of severe aplastic anemia in members who have had an insufficient response to immunosuppressive therapy.

Alvaiz

Authorization of 6 months may be granted for treatment of severe aplastic anemia in members who have had an insufficient response to immunosuppressive therapy.

MYH9-related disease with thrombocytopenia (Promacta only)

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

Myelodysplastic syndromes (Promacta only)

Authorization of 12 months may be granted for treatment of myelodysplastic syndromes (MDS).

Thrombocytopenia post-hematopoietic cell transplant

Authorization of 6 months may be granted for treatment of prolonged thrombocytopenia in members who are post-allogeneic transplant and have poor graft function.

Continuation of Therapy

Persistent or chronic immune thrombocytopenia (ITP)

- 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 dose of the requested drug for at least 4 weeks.
- 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.
- Authorization of 12 months may be granted to members with current platelet count of $50x10^9$/L to $200x10^9$/L.
- 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 dosing for the requested drug will be adjusted to achieve a platelet count sufficient to avoid clinically important bleeding.

Thrombocytopenia associated with chronic hepatitis C

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

Severe aplastic anemia

- 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 the requested drug for at least 16 weeks.
- Authorization of 12 months total may be granted to members with current platelet count less than $50x10^9$/L who are transfusion-independent.
- Authorization of 12 months may be granted to members with current platelet count of $50x10^9$/L to $200x10^9$/L.

- 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 dosing for the requested drug will be adjusted to achieve and maintain an appropriate target platelet count.

MYH9-related disease with thrombocytopenia (Promacta only)

All members (including new members) requesting authorization for continuation of therapy must meet all requirements in the coverage criteria.

Myelodysplastic syndromes (Promacta only) and thrombocytopenia post-hematopoietic cell transplant

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

Appendix

Examples of risk factors for bleeding (not all inclusive)

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

References

1. Promacta [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; March 2023.
2. Alvaiz [package insert]. Parsippany, NJ: Teva Pharmaceuticals; May 2024.
3. 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.
4. The NCCN Drugs & Biologics Compendium® © 2024 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 10, 2024.
5. The NCCN Clinical Practice Guidelines in Oncology® Myelodysplastic Syndrome (Version 2.2024). © 2024 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 10, 2024.
6. Nuenert C, Terrel DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. Blood Adv. 2019;3(23):3829–3866.

7. Provan D, Arnold DM, Bussel JB, et al. Updated international consensus report on the investigation and management of primary immune thrombocytopenia. Blood Adv. 2019;3(22): 3780–3817.
8. 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.
9. 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.
10. 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.
11. Townsley DM, Scheinberg P, Winkler T, et al. Eltrombopag added to standard immunosuppression for aplastic anemia. N Engl J Med. 2017;376:1540-1550.
12. Kulasekararaj A, Cavenagh J, Dokal I, et al. Guidelines for the diagnosis and management of adult aplastic anaemia: A British Society for Haematology guideline. Br J Haematol. 2024;204(3):784-804.