

Eltrombopag
Effective 01/01/2023

Plan	☐ MassHealth UPPL ☒ Commercial/Exchange		Program Type	☒ Prior Authorization
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit			☐ Quantity Limit ☐ Step Therapy
Specialty Limitations	This medication has been designated a specialty medication and must be filled at a contracted specialty pharmacy.			
Contact Information	Medical Benefit	Pharmacy Benefit	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A			

Overview

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

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

Coverage Guidelines

Authorization may be granted for members new to the plan who are currently receiving treatment with eltrombopag, excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

Or

Authorization may be granted when the following diagnosis specific criteria are met:

Chronic or persistent immune thrombocytopenia (ITP)

1. Member has a diagnosis of chronic or persistent ITP
2. Inadequate response or intolerance to prior therapy with corticosteroids, immunoglobulins, or splenectomy
3. Untransfused platelet count at any point prior to the initiation of the requested medication 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 Appendix A)

Thrombocytopenia associated with chronic hepatitis C

1. Member is prescribed eltrombopag for the initiation and maintenance of interferon-based therapy for the treatment of thrombocytopenia associated with chronic hepatitis C.

Aplastic anemia

1. ONE of the following:
 - a. First-line treatment of severe aplastic anemia when eltrombopag will be used in combination with standard immunosuppressive therapy (e.g., horse antithymocyte globulin (h-ATG) and cyclosporine).
 - b. Treatment of aplastic anemia which had an insufficient response to immunosuppressive therapy.

MYH9-related disease with thrombocytopenia

1. Member has a diagnosis of thrombocytopenia associated with MYH9-related disease.

Myelodysplastic Syndromes

1. Member has a diagnosis of myelodysplastic syndrome
2. ONE of the following:
 - a. 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).
 - b. 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.

Continuation of Therapy

Reauthorizations may be granted when the following clinical documentation is submitted:

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 eltrombopag 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 eltrombopag dosing 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.

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 eltrombopag 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 eltrombopag dosing will be adjusted to achieve and maintain an appropriate target platelet count.

MYH9-related disease with thrombocytopenia

All members requesting authorization for continuation of therapy must meet all initial authorization criteria.

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).

Limitations

1. Initial approvals will be granted for:
 - a. ITP, Thrombocytopenia associated with chronic Hepatitis C, and Aplastic anemia: 6 months
 - b. MYH9-related disease with thrombocytopenia, Myelodysplastic Syndrome: 12 months
2. Reauthorizations will be granted based on disease specific criteria from 3 months to 12 months.

Appendix

Appendix A: 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 member to trauma

References

1. Promacta [package insert]. Research Triangle Park, NC: GlaxoSmithKline; February 2021.
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. The NCCN Drugs & Biologics Compendium® © 2020 National Comprehensive Cancer Network, Inc. <https://www.nccn.org>. Accessed June 15, 2021.
4. The NCCN Clinical Practice Guidelines in Oncology® Myelodysplastic Syndrome (Version 2.2020). © 2020 National Comprehensive Cancer Network, Inc. <https://www.nccn.org>. Accessed June 15, 2021.
5. Nuenert C, Terrel DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. *Blood Adv* 2019;3(23):3829–3866.
6. 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.
7. 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.



8. 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.
9. 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.
10. Townsley DM, Scheinberg P, Winkler T, et al. Eltrombopag added to standard immunosuppression for aplastic anemia. *N Engl J Med* 2017;376:1540-1550.

Review History

09/21/2022 – Created for Sept P&T; switched from SGM to custom criteria. Effective 01/01/2023.

