Mass General Brigham
Health Plan

Promacta (eltrombopag)
Effective 01/01/2023

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit (NLX)		
Specialty Limitations	This medication has been designated a specialty medication and must be filled at a contracted specialty pharmacy.		
Contact Information	**Specialty Medications**		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	Non-Specialty Medications		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	Medical Specialty Medications (NLX)		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
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 Promacta, 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 $30x10^9$/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 Appendix A)

Thrombocytopenia associated with chronic hepatitis C

1. Member is prescribed Promacta 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 Promacta 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.

<u>Continuation of Therapy</u>

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 $50x10^9$/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.

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 $50x10^9$/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.

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.