Reference number(s)
1927-A

SPECIALTY GUIDELINE MANAGEMENT

NPLATE (romiplostim)

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. Nplate is indicated for the treatment of thrombocytopenia in:
      a. Adult patients with immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
      b. Pediatric patients 1 year of age and older with ITP for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
   2. Nplate is indicated to increase survival in adults and in pediatric patients (including term neonates) acutely exposed to myelosuppressive doses of radiation (Hematopoietic Syndrome of Acute Radiation Syndrome [HSARS]).

B. Compendial Uses
   1. Myelodysplastic syndromes, for lower risk disease in patients with severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents, immunosuppressive therapy, or clinical trial
   2. Chemotherapy-induced thrombocytopenia (CIT)

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. Immune thrombocytopenia: pretreatment and current platelet counts
B. Chemotherapy-induced thrombocytopenia (CIT): pretreatment and current platelet counts

III. EXCLUSIONS

Coverage will not be provided for members with the following exclusion: concomitant use of Nplate with other thrombopoietin receptor agonists (e.g., Promacta, Doptelet, Mulpleta) or with spleen tyrosine kinase inhibitors (e.g., Tavalisse)

IV. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a hematologist or oncologist.

V. CRITERIA FOR INITIAL APPROVAL

A. Immune thrombocytopenia (ITP)

Authorization of 6 months may be granted for treatment of 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 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 Section VII).

B. Hematopoietic syndrome of acute radiation syndrome (HSARS)

Authorization of 1 month may be granted for treatment of hematopoietic syndrome of acute radiation syndrome (acute exposure to myelosuppressive doses of radiation).

C. 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), immunosuppressive therapy, or clinical trial.

D. Chemotherapy-induced thrombocytopenia

Authorization of 6 months may be granted for treatment of chemotherapy-induced thrombocytopenia (CIT) when any of the following criteria are met:

1. The platelet count is less than $100x10^9$/L for at least 3-4 weeks following the last chemotherapy administration, or
2. Chemotherapy administration has been delayed related to thrombocytopenia

VI. CONTINUATION OF THERAPY

A. Immune thrombocytopenia (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 Nplate 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 Nplate dosing will be adjusted to achieve a platelet count sufficient to avoid clinically important bleeding.

B. Hematopoietic syndrome of acute radiation syndrome (HSARS)

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

C. 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).

D. Chemotherapy-induced thrombocytopenia
Authorization of 6 months may be granted for continued treatment of chemotherapy-induced thrombocytopenia (CIT) in members who experience benefit from therapy (e.g., increased platelet counts, decreased bleeding events, reduced need for platelet transfusions) to maintain a target platelet count goal of $100x10^9$/L – $200x10^9$/L.

VII. 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 member to trauma

VIII. REFERENCES

1. Nplate [package insert]. Thousand Oaks, CA: Amgen Inc.; February 2022.
2. The NCCN Drugs & Biologics Compendium® © 2020 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 15, 2021.
3. The NCCN Clinical Practice Guidelines in Oncology® Myelodysplastic Syndrome (Version 2.2020). © 2021 National Comprehensive Cancer Network, Inc. https://www.nccn.org. Accessed June 15, 2021.
4. Nuenert C, Terrel DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. *Blood Adv* 2019;3(23):3829–3866.
5. 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.
6. 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.