CARECONTINUUM™

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Thrombocytopenia – Nplate Utilization Management Medical Policy
- Nplate® (romiplostim subcutaneous injection – Amgen)

REVIEW DATE: 04/23/2025; selected revision 05/07/2025

OVERVIEW

Nplate, a thrombopoietin receptor agonist, is indicated for the treatment of:[1]

- **Hematopoietic syndrome of acute radiation syndrome**, to increase survival in adults and pediatric patients (including term neonates) acutely exposed to myelosuppressive doses of radiation.
- **Immune thrombocytopenia (ITP), in adults** who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
- **Immune thrombocytopenia (ITP), in pediatric patients ≥ 1 year of age** with ITP for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.

Nplate should only be utilized in patients with ITP whose degree of thrombocytopenia and clinical condition increase the risk for bleeding.[1] Nplate should not be used in an attempt to normalize platelet counts.

Guidelines

Nplate is mentioned in various clinical guidelines.

- **Chemotherapy Induced Thrombocytopenia:** The National Comprehensive Cancer Network (NCCN) guidelines for hematopoietic growth factors (version 1.2025 – October 11, 2024) recommend consideration of Nplate for the management of suspected chemotherapy induced thrombocytopenia (category 2A) in addition to other modalities (e.g., platelet transfusion, chemotherapy dose reduction, or change in treatment regimen).[14]
- **Immune Thrombocytopenia:** The American Society of Hematology has updated guidelines for ITP (2019). For adults with ITP for at least 3 months who are corticosteroid-dependent or unresponsive to a corticosteroid, a thrombopoietin receptor agonist (Nplate or Promacta® [eltrombopag tablets and oral suspension]) or a splenectomy are recommended.[2] In children with newly diagnosed ITP who have non-life-threatening mucosal bleeding, corticosteroids are recommended. For children who have non-life-threatening mucosal bleeding and do not respond to first-line treatment, thrombopoietin receptor agonists are recommended.
- **Myelodysplastic Syndrome (MDS):** NCCN recommendations regarding MDS (version 2.2025 – January 17, 2025) state to consider treatment with a thrombopoietin receptor agonist in patients with lower-risk MDS who have severe or life-threatening thrombocytopenia.[3] Data are available that describe the use of Nplate in patients with MDS.[4-13] The data with Nplate are discussed noting an increased rate of platelet response and decreased overall bleeding events among patients with low to intermediate risk MDS.
- **Thrombocytopenia in a Patient Due to Immune Checkpoint Inhibitor Therapy:** NCCN guidelines for the management of immunotherapy-related toxicities (version 1.2025- December 20, 2024) recommend Nplate as one of the agents to consider if the patient has a platelet count ≤ 50,000/mm^3 and has not had a response to systemic corticosteroids after 1 to 2 weeks.[15]

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Nplate. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indications. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Regarding the approval duration of one dose, the approval is for 30 days, which is an adequate duration for the patient to receive one dose. Because of the specialized skills required for evaluation and diagnosis of patients treated with Nplate as well as the monitoring required for adverse events and long-term efficacy, approval for some indications requires Nplate to be prescribed by or in consultation with a physician who specializes in the condition being treated.

*Indications and/or approval conditions noted with **[eviCore]** are managed by eviCore healthcare for those clients who use eviCore for oncology and/or oncology-related reviews. For these conditions, a prior authorization review should be directed to eviCore at www.eviCore.com.*

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Nplate is recommended in those who meet ONE of the following criteria:

FDA-Approved Indications

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1. **Hematopoietic Syndrome of Acute Radiation Syndrome.** *[eviCore]* Approve for one dose if the patient has been acutely exposed to myelosuppressive doses of radiation.

   Dosing. Approve up to 10 mcg/kg administered subcutaneously given once.

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2. **Immune Thrombocytopenia.** Approve if the patient meets ONE of the following (A or B):
   - **A)** Initial Therapy. Approve for 3 months if the patient meets ALL of the following (i, ii, and iii):
     - **i.** Patient meets ONE of the following (a or b):
       - **a)** Patient has a platelet count < 30 x 10^9/L (< 30,000/mcL); OR
       - **b)** Patient meets BOTH of the following [(1) and (2)]:
         - **(1)** Patient has a platelet count < 50 x 10^9/L (< 50,000/mcL); AND
         - **(2)** According to the prescriber the patient is at an increased risk of bleeding; AND
     - **ii.** Patient meets ONE of the following (a or b):
       - **a)** Patient has tried at least one other therapy; OR
         Note: Examples of therapies are systemic corticosteroids, intravenous immunoglobulin, anti-D immunoglobulin, Promacta (eltrombopag tablets and oral suspension), Tavalisse (fostamatinib tablets), Doptelet (avatrombopag tablets) and ritixumab.
       - **b)** Patient has undergone splenectomy; AND
     - **iii.** Medication is prescribed by or in consultation with a hematologist; OR
   - **B)** Patient is Currently Receiving Nplate. Approve for 1 year if the patient meets BOTH of the following (i and ii):
     - **i.** According to the prescriber the patient demonstrates a beneficial clinical response; AND
       Note: A beneficial response can include increased platelet counts, maintenance of platelet counts, and/or a decreased frequency of bleeding episodes.

ii. Patient remains at risk for bleeding complications.

Dosing. Approve up to 10 mcg/kg subcutaneously no more frequently than once weekly.

Other Uses with Supportive Evidence

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3. Thrombocytopenia, Chemotherapy Induced. ***[eviCore]*** Approve if the patient meets ONE of the following (A or B):

A) Initial Therapy. Approve for 3 months if the patient meets ALL the following (i, ii, iii, and iv):
- **i.** Patient is ≥ 18 years of age; AND
- **ii.** Patient has a platelet count < 100 x 10^9/L (< 100,000/mcL); AND
- **iii.** Patient meets ONE of the following (a or b):
  - **a)** Patient has thrombocytopenia at least 3 weeks after the most recent dose of chemotherapy; OR
  - **b)** Patient has experienced a delay in chemotherapy administration related to thrombocytopenia; AND
- **iv.** Medication is prescribed by or in consultation with a hematologist or an oncologist; OR

B) Patient is Currently Receiving Nplate. Approve for 6 months if the patient meets the ALL of following (i, ii, and iii):
- **i.** Patient is ≥ 18 years of age; AND
- **ii.** Patient continues to receive treatment with chemotherapy; AND
- **iii.** Patient demonstrates a beneficial clinical response according to the prescriber.
  Note: A beneficial response can include increased platelet counts, maintenance of platelet counts, and/or decreased frequency of bleeding episodes.

Dosing. Approve up to 10 mcg/kg subcutaneously no more frequently than once weekly.

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4. Thrombocytopenia in Myelodysplastic Syndrome. ***[eviCore]*** Approve if the patient meets ONE of the following criteria (A or B):

A) Initial Therapy. Approve for 3 months if the patient meets ALL the following (i, ii, and iii):
- **i.** Patient has low- to intermediate-risk myelodysplastic syndrome; AND
- **ii.** Patient meets ONE of the following (a or b):
  - **a)** Patient has a platelet count < 30 x 10^9/L (< 30,000/mcL); OR
  - **b)** Patient meets BOTH of the following [(1) and (2)]:
    - **(1)** Patient has a platelet count < 50 x 10^9/L (< 50,000/mcL); AND
    - **(2)** According to the prescriber the patient is at an increased risk for bleeding; AND
- **iii.** Medication is prescribed by or in consultation with a hematologist or an oncologist; OR

B. Patient is Currently Receiving Nplate. Approve for 1 year if the patient meets BOTH of the following (i and ii):
- **i.** According to the prescriber the patient demonstrates a beneficial clinical response; AND
  Note: An example of a response is increased platelet counts, maintenance of platelet counts, and/or decreased frequency of bleeding episodes.
- **ii.** Patient remains at risk for bleeding complications.

Dosing. Approve up to 1,500 mcg subcutaneously no more frequently than twice weekly.

5. Thrombocytopenia in a Patient Due to Immune Checkpoint Inhibitor Therapy. *[eviCore]*
Approve for 6 months if the patient meets ONE of the following (A or B):
Note: Examples of checkpoint inhibitors are Keytruda (pembrolizumab intravenous infusion), Opdivo (nivolumab intravenous infusion), Yervoy (ipilimumab intravenous infusion), Tecentriq (atezolizumab intravenous infusion), Bavencio (avelumab intravenous infusion), Imfinzi (durvalumab intravenous infusion), and Libtayo (cemiplimab-rwlc intravenous infusion).

A) Initial Therapy. Approve if the patient meets ALL the following (i, ii, and iii):
- **i.** Patient has tried at least one systemic corticosteroid; AND
  Note: Examples of a corticosteroid include methylprednisolone and prednisone.
- **ii.** Patient has a platelet count < 50 x 10^9/L (< 50,000/mcL); AND
- **iii.** The medication is prescribed by or in consultation with a hematologist or an oncologist; OR

B) Patient is Currently Receiving Nplate. Approve if according to the prescriber, the patient demonstrated a beneficial clinical response.
Note: A beneficial response can include increased platelet counts, maintenance of platelet counts, and/or decreased frequency of bleeding episodes.

Dosing. Approve up to 1,500 mcg subcutaneously no more frequently than twice weekly.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Nplate is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

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4. Giagounidis A, Mufti GJ, Fenaux P, et al. Results of a randomized, double-blind study of romiplostim versus placebo in patients with low/intermediate-1-risk myelodysplastic syndrome and thrombocytopenia. *Cancer*. 2014;120:1838-1846.
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7. Fenaux P, Muus P, Kantarjian H, et al. Romiplostim monotherapy in thrombocytopenia patients with myelodysplastic syndromes: long-term safety and efficacy. *Br J Haematol*. 2017;178:906-913.
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10. Wang ES, Lyons RM, Larson RA, et al. A randomized, double-blind, placebo-controlled phase 2 study evaluating the efficacy and safety of romiplostim treatment of patients with low or intermediate-1 risk myelodysplastic syndrome receiving lenalidomide. *J Hematol Oncol*. 2012;5:71.
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12. Brierley CK Steensma DP. Thrombopoiesis-stimulating agents and myelodysplastic syndromes. *Br J Haematol*. 2015;169:309-323.
13. Prica A, Sholzberg M, Buckstein R. Safety and efficacy of thrombopoietin-receptor agonists in myelodysplastic syndromes: a systematic review and meta-analysis of randomized controlled trials. *Br J Haematol*. 2014;167:626-638.

14. The NCCN Hematopoietic Growth Factors Clinical Practice Guidelines in Oncology (Version 1.2025 – October 11, 2024). © 2024 National Comprehensive Cancer Network. Available at: http://www.nccn.org. Accessed on April 18, 2025.
15. The NCCN Management of Immunotherapy-Related Toxicities (Version 1.2025 – December 20, 2024). © 2025 National Comprehensive Cancer Network. Available at: http://www.nccn.org. Accessed on April 18, 2025.

HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	04/12/2023
Annual Revision	**Hematopoietic Syndrome of Acute Radiation Syndrome:** It was added that this indication will be routed to Evicore. **Thrombocytopenia, Chemotherapy Induced:** It was added that this indication will be routed to Evicore. **Thrombocytopenia in Myelodysplastic Syndrome:** It was added that this indication will be routed to Evicore.	04/24/2024
Annual Revision	**Thrombocytopenia in a Patient Due to Immune Checkpoint Inhibitor Therapy:** This condition and criteria for approval were added to the policy for Nplate, as well as Dosing. This indication will be routed to Evicore.	04/23/2025
Selected Revision	**Thrombocytopenia in a Patient Due to Immune Checkpoint Inhibitor Therapy:** For Initial Therapy, the criteria that the patient has not had a response to at least one systemic corticosteroid was changed to the patient has tried at least one systemic corticosteroid.	05/07/2025