Medical Drug Clinical Criteria

Subject:	Nplate (romiplostim)		
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Overview

This document addresses the use of Nplate (romiplostim). Nplate is a thrombopoietin (TPO) receptor agonists primarily used in the treatment of children and adults with immune thrombocytopenia, an autoimmune disorder that can cause uncontrolled bleeding if left untreated.

Immune thrombocytopenia (ITP) is also called idiopathic thrombocytopenia purpura and immune thrombocytopenia purpura, which is an acquired autoimmune disorder characterized by low platelet counts caused by autoantibodies against platelet antigens. According to the National Institutes of Health, ITP occurs in approximately 1 in every 16,000 adults, causing unusual bruising or bleeding due to an abnormally low number of platelets in the blood. Nplate is FDA approved for the treatment of thrombocytopenia in individuals with ITP who had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.

Nplate is FDA indicated for the following:

- Adult patients with immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
- 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.
- Adults and pediatrics (including term neonates) acutely exposed to myelosuppressive doses of radiation (Hematopoietic Syndrome of Acute Radiation Syndrome [HS-ARS].

Limitations of Use per label:

- Nplate is not indicated for the treatment of thrombocytopenia due to myelodysplastic syndrome (MDS) or any cause of thrombocytopenia other than ITP
- Nplate should be used only in patients with ITP whose degree of thrombocytopenia and clinical condition increases the risk for bleeding
- Nplate should not be used in an attempt to normalize platelet counts.

Per specialty committee consensus opinion, ongoing treatment with Nplate (romiplostin) may be used to maintain an adequate platelet count (50 – 100 X 10^9/L) to decrease the risk of bleeding. For platelet count greater than 100,000/mm^3, dose adjustments can be made using a cut-off platelet level of 100,000/mm^3 as a substitute for 200,000/mm^3 in the FDA dosage and administration recommendations.

The NCCN Drugs and Biologics Compendium and NCCN Clinical Practice Guideline offers a Category 2A recommendation for the treatment of individuals with lower risk MDS disease with severe or refractory thrombocytopenia using romiplostim following disease progression or no response to hypomethylating agents, immunosuppressive therapy, or clinical trial. “Lower risk defined as IPSS-R (Very Low, Low, Intermediate), IPSS (Low/Intermediate-1), WPSS (Very low, low, intermediate)”. NCCN also provides a 2A recommendation for use of Nplate in chemotherapy-induced thrombocytopenia with the goal of allowing resumption of chemotherapy regimen when the benefits outweigh the risks.

Definitions and Measures

Aplastic anemia: A condition that occurs when the body stops producing enough new blood cells.

Immune thrombocytopenia: A bleeding disorder where the blood is unable to clot, as a result of a low number of platelets or thrombocytes.

The International Prognostic Scoring System (IPSS): IPSS is the most widely used prognostic classification system for myelodysplastic syndrome (MDS). The IPSS-R is the revised international prognostic scoring system in MDS to better predict outcomes in newly

diagnosed patients. The WHO classification-based Prognostic Scoring System (WPSS) allows for dynamic estimation of prognosis at multiple time points during the course of MDS.

Maintenance therapy: Designed to maintain a condition to prevent a relapse.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Nplate (romiplostim)

Initial requests for Nplate (romiplostim) for **ITP** may be approved if the following criteria are met:

I. Individual has a diagnosis of immune (idiopathic) thrombocytopenia (ITP) and the following are met:
   A. Documentation is provided that individual has a platelet count of less than 30 x 10^9/L or active bleeding (ASH, 2011; Hicks et al., 2014); **AND**
   B. Individual has had a prior trial and insufficient response to **one** of the following:
      1. Corticosteroids; **OR**
      2. Immunoglobulins (for example IVIg or anti-D); **OR**
      3. Splenectomy;

Continuation requests for Nplate (romiplostim) for **ITP** may be approved if the following criteria are met:

I. Individual has a diagnosis of ITP and the following are met:
   A. Documentation is provided that individual has demonstrated a response to therapy as shown by increased platelet counts; **AND**
   B. Continuation of treatment is to maintain an adequate platelet count (50 – 100 X 10^9/L)* to decrease the risk of bleeding.

Approval Duration for ITP:
Initial requests: 6 months
Continuation requests: 12 months

Initial requests for Nplate (romiplostim) for **MDS** may be approved if the following criteria are met:
I. Documentation is provided that individual has a diagnosis of lower risk myelodysplastic syndrome (MDS) [Lower risk defined as IPSS-R (Very Low, Low, Intermediate), IPSS (Low/Intermediate-1), WPSS (Very low, low, intermediate)] (NCCN 2A); **AND**
II. Individual has severe or refractory thrombocytopenia following disease progression or no response to hypomethylating agents or immunosuppressive therapy.

Continuation requests for Nplate (romiplostim) for **MDS** may be approved if the following criteria are met (NCCN 2A):

I. Documentation is provided that individual has demonstrated a clinically significant response to therapy, such as an increase in platelet counts, decrease in bleeding events, or reduction in need for platelet transfusions.

Approval Duration for MDS:
Initial requests: 6 months
Continuation requests: 12 months

Requests for Nplate (romiplostim) for **HS-ARS** may be approved if the following criteria are met:

I. Individual a diagnosis of Hematopoietic Syndrome of Acute Radiation Syndrome [HS-ARS] (i.e., acute exposure to myelosuppressive doses of radiation); **AND**
II. Individual has exposure or suspected exposure to radiation levels greater than 2 gray (Gy).

Approval **Duration for HS-ARS:** 1 single administration per episode

Initial requests for Nplate (romiplostim) for **CIT** may be approved if the following criteria are met (NCCN 2A):
I. Individual has a diagnosis of chemotherapy-induced thrombocytopenia (CIT); **AND**
II. Individual meets one of the following criteria:
   A. Individual has platelets less than 100 x 10^9/L for at least 3 weeks following the last chemotherapy administration; **OR**
   B. Individual has platelets less than 100 x 10^9/L and there are delays in chemotherapy related to thrombocytopenia; **AND**
III. Individual was using a cytotoxic chemotherapy agent that is known to cause thrombocytopenia; **AND**

IV. The goal of therapy is to maintain the dosing schedule and/or intensity of the chemotherapy regimen when such benefit outweighs the potential risks.

Continuation requests for Nplate (romiplostim) for **CIT** may be approved if the following criteria are met:

I. Individual has a diagnosis of CIT and the following are met:
   A. Individual has demonstrated a response to therapy shown by increased platelet counts; **AND**
   B. Continuation of treatment is to maintain an adequate platelet count (100 - 150 X 10^9/L) to allow for the resumption of chemotherapy regimen as appropriate.

Approval Duration for CIT:
Initial requests: 6 months
Continuation requests: 12 months

Initial requests for Nplate (romiplostim) for **immunotherapy-related thrombocytopenia** may be approved if the following criteria are met (NCCN 2A):

I. Documentation is provided that individual has a diagnosis of immunotherapy-related Grade 3 (G3) or Grade 4 (G4) thrombocytopenia (defined as platelet count 50000/mm^3 or less); **AND**
II. Thrombocytopenia is due to immune checkpoint inhibitor therapy; **AND**
III. Thrombocytopenia has not responded to corticosteroid treatment after at least one (1) week of therapy.

Continuation requests for Nplate (romiplostim) for **immunotherapy-related thrombocytopenia** may be approved if the following criteria are met:

I. Individual has a diagnosis of immunotherapy-related thrombocytopenia; **AND**
II. Documentation is provided that individual has demonstrated a response to therapy shown by increased platelet counts.

Approval Duration for immunotherapy-related thrombocytopenia:
Initial requests: 6 months
Continuation requests: 12 months

Nplate (romiplostim) may not be approved for the following:
I. Individual is using to normalize platelet counts; **OR**
II. Individual is requesting for the treatment of low platelet count caused by any condition other than those conditions listed above; **OR**
Individual is using in combination with eltrombopag (Alvaiz or Promacta); **OR**
III. When the above criteria are not met and for all other indications.

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J2802	Injection, romiplostim, 1 mcg [Nplate]

ICD-10 Diagnosis

D46.0-D46.9	Myelodysplastic syndromes
D69.3	Immune thrombocytopenic purpura
D69.49	Other primary thrombocytopenia
D69.59	Other secondary thrombocytopenia
T45.1X5A-T45.1X5S	Adverse effect of antineoplastic and immunosuppressive drugs
T66.XXXA--T66.XXXS	Radiation sickness, unspecified

Document History

Revised: 05/16/2025

Document History:

- 05/16/2025 – Annual Review: Add immunotherapy-related thrombocytopenia. Coding Reviewed: Added ICD-10-CM T45.1X5A-T45.1X5S, added T66.XXXD and T66.XXXS and updated description for range T66.XXXA-T66.XXXS. Removed ICD-10-CM D69.41 and D69.42.
- 11/25/24 – CMS Coding Update: Add HCPCS J2802 effective 1/1/25 and delete J2796.
- 05/17/2024 – Annual Review: update do not approve criteria, wording or formatting. Coding Reviewed: Added ICD-10-CM T66.XXXA, D69.59 (Effective 05/21/2021).
- 05/19/2023 – Annual Review: remove notes from criteria. Coding Reviewed: No changes.
- 05/20/2022 – Annual Review: Clarify do not approve criteria. Coding Reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 05/21/2021 – Annual Review: Update criteria to add approval durations for ITP and MDS indications. Update MDS criteria to add continuation request parameters. Clarify use in low risk MDS. Update criteria to add new indication for HS-ARS per label. Update criteria to add new NCCN recommendation for CIT. Wording and formatting updates. Coding Reviewed: No changes.
- 05/15/2020 – Annual Review: Update criteria to remove requirement for "chronic" ITP per FDA label update. Update non-approvable criteria for consistency. Coding Reviewed: No changes.
- 05/17/2019 – Annual Review: Initial review of Nplate. Minor wording and formatting changes. Coding reviewed: No changes.

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