OVERVIEW:
Nplate, a thrombopoietin receptor agonist, is indicated for the treatment of thrombocytopenia in patients with chronic immune thrombocytopenia purpura (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Some limitations of use are that Nplate is not indicated for the treatment of thrombocytopenia due to myelodysplastic syndrome (MDS) or any cause of thrombocytopenia other than chronic ITP. Nplate should only be utilized in patients with ITP whose degree of thrombocytopenia and clinical condition increase the risk for bleeding. Nplate should not be used in an attempt to normalize platelet counts. The initial Nplate dose is 1 mcg/kg once weekly as a subcutaneous (SC) injection by a healthcare provider. The dose should be adjusted weekly by increments of 1 mcg/kg to achieve and maintain a platelet count ≥ 50 x 10^9/L as needed to reduce the bleeding risk. Do not exceed a maximum weekly dose of 10 mcg/kg. Do not dose if the platelet count is > 400 x 10^9/L. Discontinue Nplate if the platelet count does not increase after 4 weeks at the maximum dose. Nplate contains a Warning that in clinical trials with Nplate progression from MDS to acute myelogenous leukemia (AML) has been observed. Also, there is a Warning that thrombotic/thromboembolic complications may occur due to increases in platelet counts with Nplate therapy.

POLICY STATEMENT
Prior authorization is recommended for prescription benefit coverage of Nplate. All approvals are provided for 1 year in duration unless otherwise noted below. In cases where the approval is authorized in months, 1 month is equal to 30 days.

Automation: None.

Recommended Authorization Criteria
Coverage of Nplate is recommended in those who meet the following criteria:

Food and Drug Administration (FDA)-Approved Indications

1. Treatment of Thrombocytopenia in Patients with Chronic Immune (Idiopathic) Thrombocytopenia Purpura (ITP). Approve Nplate if the patient meets the following criteria (a, b, c, and d):
   a. The agent is prescribed by, or in consultation with, a hematologist; AND
   b. The patient is ≥ 18 years of age; AND
   c. Not using Nplate in combination with Promacta; AND
   d. The patient meets ONE of the following conditions (i, ii, iii, or iv):
      i. The patient is not a candidate for corticosteroids, IVIG or a splenectomy; OR
      ii. The patient has tried corticosteroids; OR
      iii. The patient has tried IVIG; OR
      iv. The patient has undergone a splenectomy.

Nplate is indicated for the treatment of thrombocytopenia in patients with chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. The safety and efficacy
of Nplate in pediatric patients (aged < 18 years) have not been established. The pivotal trials with Nplate involved patients who had tried at least one primary ITP therapy (e.g., corticosteroids, immunoglobulins); approximately 50% of patients had undergone splenectomy. Evidence-based practice guidelines for immune thrombocytopenia from ASH (published in 2011), recommend corticosteroids or IVIG as first-line treatment for adults; splenectomy is recommended for patients who have failed corticosteroid therapy. Thrombopoietin receptor agonists are recommended for adults at risk of bleeding who relapse following splenectomy or who have a contraindication to splenectomy and who have failed at least one other therapy. At this time recommendations for use of thrombopoietin receptor agonists in children with ITP cannot be made; clinical trials have been initiated. Trials with Nplate in children are evolving.

2. **Patient Has Been Started on Nplate.** Approve for an indication or condition addressed as an approval in the Recommended Authorization Criteria section (FDA-Approved Indications) if the patient meets the following criteria (a and b):

   a. Patient continues to respond to therapy with this drug (e.g. platelet count has increased); AND

**Other Uses with Supportive Evidence**

1. **Thrombocytopenia in Myelodysplastic Syndrome (MDS).** Approve Nplate for 12 months if the patient meets the following criteria (A, B and C):
   A) The agent is prescribed by, or in consultation with, a hematologist or an oncologist; AND
   B) The patient has low- to intermediate-risk MDS; AND
   C) According to the prescribing physician the patient has clinically significant thrombocytopenia (e.g., low platelet counts \(< 30,000 \text{ mm}^3\) \{pretreatment\}); is platelet transfusion-dependent; active bleeding; and/or a history of bleeding at low platelet counts).

**Conditions Not Recommended for Approval**

Nplate has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. Rationale for non-coverage for these specific conditions is provided below. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval.)

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**


**Other References Utilized**